COMPARING TWO MEANS IN COUNT MODELS HAVING RANDOM EFFECTS—A UMPU TEST

by

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Abstract

We propose a model for bivariate count data that includes a common random effect. Our interest is in rare events and our model specifies that, conditional on the random effects, the marginal distributions consist of independent Poisson distributions. Uniformly Most Powerful Unbiased tests are derived for comparing the unconditional means of the two component count distributions for a variety of random effects distributions such as lognormal, log Cauchy, gamma distribution. The optimal test turns out to be the standard binomial test obtained by conditioning on the total number of events from both components. A numerical calculation is performed to compare the power of the proposed test and the likelihood test under the various conditions.

Keywords: Uniformly Most Powerful Unbiased test, random effect, likelihood ratio test.

1 Introduction

In many longitudinal studies, subjects are only observed at a fixed number of specified time points. Consequently, the exact occurrence times of events are not available. All inferences must be made from the number of times a recurrent event occurs between successive observation times for a subject. Our emphasis is on rare events when there are a small or moderate number of subjects. In this setting, exact methods are needed which are not sensitive to the assumed form of the random effects distribution. In this paper, we consider bivariate count data generated from pairs of dichotomous observations whose components are influenced by a common random effect. More particularly, conditioned on the random effects, they are assumed to be independently distributed as Poisson variates. For example, consider a pair of the counts such as the number of epileptic seizures in the same patient for two equally spaced successive time intervals (before and after treatment). The expected mean numbers of seizures will differ from person to person depending on the length of intervals.

In section 2, we consider the case when the sum of the two, unconditional, component means is the same for all subjects. We show that the binomial test is not UMPU test under this assumption. In section 3, we enrich the model to allow the sum of the means to be different for each subject and derive uniformly most powerful unbiased (UMPU) tests for comparing two unconditional means. We require that the characteristic function of the natural logarithm of random effects does not vanish. This family of distributions for random effects contains a family of infinitely divisible distributions for the natural logarithm of random effects. This is sensible when the random effects can be regarded as a sum of small random variables where each random variable only makes a small contribution to the sum. When there are no random effects, UMPU tests are derived using the approach in Lehmann and Scheffé (1955). We prove that these tests remain UMPU even when random effects are present. In section 4, a numerical calculation is performed to compare the powers of the
proposed test and the likelihood ratio test under various conditions.

Our optimal tests are conditional, and in the examples given in Section 4, the Type I error can be maintained at its nominal level. Our main result states that, under the enriched model, the optimal test is the standard binomial test obtained by conditioning on the total number of events for the two components. It is thus easy to apply.

2 A random effects model for counts

Let $Y_{ij} (i = 1, \ldots, I, j = 1, 2)$ be independent Poisson random variables with means $E(Y_{ij}) = v_i \mu_j$ conditional on the random effects $V_i = v_i$ for all $i$. We allow the pair of counts $(Y_{i1}, Y_{i2})$ to depend on a random effect $V_i$ or possibly on the whole collection of random effects $V = (V_1, \ldots, V_I)'$. The joint probability mass function of $\bar{Y} = (Y_{11}, Y_{12}, \ldots, Y_{I1}, Y_{I2})'$ is

$$f(y) = E_V \left[ \prod_{i=1}^{I} \frac{e^{-V_i \mu_j} (V_i \mu_j)^{y_{i1}}}{y_{i1}!} \cdot \frac{e^{-V_i \mu_2} (V_i \mu_2)^{y_{i2}}}{y_{i2}!} \right]$$

$$= \frac{h(y_{1*}, \ldots, y_{I*}; \tau)}{\prod_{i=1}^{I} y_{i1}! y_{i2}!} p^{y_{i*}} (1 - p)^{y_{i*}}$$

where $p = \mu_1 / (\mu_1 + \mu_2)$, $\tau = \mu_1 + \mu_2$, $y_{i*} = y_{i1} + y_{i2}$, $y_{*j} = \sum_{i=1}^{I} y_{ij}$, $y_{**} = \sum_{i=1}^{I} \sum_{j=1}^{2} y_{ij}$,

$$h(y_{1*}, \ldots, y_{I*}; \tau) = E_V \left[ e^{-\sum_{i=1}^{I} \tilde{V}_i} \prod_{i=1}^{I} \tilde{V}_i^{y_{i*}} \right]$$

and $\tilde{V}_i = V_i \cdot \tau$.

After the reparameterization from $(\mu_1, \mu_2)$ to $(p, \tau)$ as in Nelson (1985), the parameter space is $\Theta = \{(p, \tau) : 0 < p < 1, \tau > 0\}$. The $Y_{ij}$'s are independent Poisson random variables with mean $v_i \mu_j$ $(p_1 = p, p_2 = 1 - p)$ conditional on the random effects $V = v$ where $E(\tilde{V}_i) = \tau$. We set $U = Y_{*,1}$, $T_i = Y_{i*}$ for all $i$ and $T = (T_1, \ldots, T_I)'$. From (1), $(U, T)$ is sufficient for $(p, \tau)$. The joint distribution of $(U, T)$ is given by the following theorem.

**Theorem 2.1.** The joint probability mass function of $(U, T)$ is

$$f(u; t; p, \tau) = \frac{t_{*}!}{u!(t_{*} - u)!} p^u (1 - p)^{t_{*} - u} \cdot \frac{h(t; \tau) t_{*}!}{H(t, \tau) \prod_{i=1}^{I} t_i!} \cdot \frac{H(t_{*}, \tau)}{t_{*}!}$$

$$= g_1(u; p|t_{*}) \cdot g_2(t; \tau|t_{*}) \cdot g_3(t_{*}; \tau)$$

$$= \frac{t_{*}!}{u!(t_{*} - u)!} p^u (1 - p)^{t_{*} - u} \cdot \frac{h(t; \tau)}{\prod_{i=1}^{I} t_i!}$$

$$= g_1(u; p|t) \cdot g_3(t; \tau)$$

where $T_{*} = \sum_{i=1}^{I} T_i$ and $H(t, \tau) = \tau^{t_{*}} E_V \left[ e^{-\tau \sum_{i=1}^{I} V_i (\sum_{i=1}^{n} V_i)^{t_{*}}} \right] = E_{\tilde{V}} \left[ e^{-\sum_{i=1}^{I} \tilde{V}_i (\sum_{i=1}^{n} V_i)^{t_{*}}} \right]$.

**Proof:** See Appendix.

We need two auxiliary results for our development. The following theorem is a multivariate version of the $\tau E$ in Goldberg (1962).
Lemma 2.1. Let $y = (y_1, \ldots, y_I)^t$, $x = (x_1, \ldots, x_I)^t$ and $\psi(y) = \int_{R} f(y-x)g(x)dx$ where $R = (-\infty, \infty) \times \cdots \times (-\infty, \infty)$. If $f$ and $g$ are absolutely integrable on $R$, then the Fourier transformation of $\psi$ is $c_\psi(\omega) = \int \psi(y)e^{-i\omega^t y}dy = c_f(\omega)c_g(\omega)$, where $\omega = (\omega_1, \ldots, \omega_I)^t$, $c_f(\omega) = \int f(x)e^{-i\omega^t x}dx$, and $c_g(\omega) = \int g(x)e^{-i\omega^t x}dx$.

Proof: See Appendix.

Lemma 2.2. Let $F_X(x) = \int_{R_X} f(u)du$ with $x = (x_1, \ldots, x_I)^t$, $R_X = (-\infty, x_1) \times \cdots \times (-\infty, x_I)$ and let $P_X^{\eta}$ be the probability distribution associated with a probability density function $f(x - \eta)$ where $\{\eta_i : -\infty < \eta_i < \infty\}$, $i = 1, \ldots, I$. Then its characteristic function $\varphi(s)$ does not vanish for any $s$ if and only if the location family $P_X^{\eta}$ is boundedly complete.

Proof: See Appendix.

This is a multivariate version of Theorem 2.4 in Ghosh and Singh (1960). It deals with the location family such that the characteristic function for $X$ does not vanish. Such a family of distributions contains a family of infinitely divisible distributions such as a normal distribution with known variances and a Cauchy distribution with known scale parameters. Accordingly, we make the following assumption for the distribution of random effects.

Assumption 1. The characteristic function of $X = \log V$, $\varphi(s) = E[\exp(is^t X)]$ does not vanish for any $s$.

This includes a variety of random effects distributions such as the lognormal, log Cauchy, and gamma distribution.

Theorem 2.2. Under Assumption 1,
(i) The family $P^{T^*_i}$ of distributions $P^{T^*_i}$ for $T^*_i$ is boundedly complete. Hence $T^*_i$ is not sufficient, but complete.
(ii) The family $P^T$ of distributions $P^T$ for $T$ is not boundedly complete. Hence $T$ is sufficient, but not complete.

Proof: See Appendix.

Therefore, we may not be able to obtain UMP unbiased tests. To have boundedly completeness for the full vector $T$, we need a rich enough family of the distributions. Therefore, we enrich the family of random effects by allowing the marginal distribution of $V_i$ to depend on $i$.

3 A UMPU test under an enriched random effects model

In this section, we show the standard binomial tests are still uniformly most powerful unbiased tests under an enriched family of distributions for random effects. To obtain this
family, we replace $\tau$ in (1) by $\tau_i = E(\tilde{V}_i) = E(V_i \tau_i)$. Then the $Y_{ij}$ \((i = 1, \ldots, I, j = 1, 2)\) are independent Poisson random variables with means $E(Y_{ij}) = \tilde{v}_i p_j \ (p_1 = p, p_2 = 1 - p)$ conditional on the random effects $\tilde{V}_i = \tilde{v}_i$ satisfying Assumption 1. The parameter space is now $\Theta = \{(p, \tau_1, \ldots, \tau_I) : 0 < p < 1, \tau_i > 0, i = 1, \ldots, I\}$.

The joint probability mass function is then

$$f(\bar{y}) = \frac{h(y_{1*}, \ldots, y_{I*}; \bar{\tau})}{\prod_{i=1}^{I} y_{i1}! y_{i2}!} p_{y_{1*}}^\tau (1 - p)^{y_{2*}}$$

where

$$\tau = (\tau_1, \ldots, \tau_I)', \quad h(y_{1*}, \ldots, y_{I*}; \tau) = E_{\hat{V}} \left[e^{-\sum_{i=1}^{I} \hat{V}_i} \prod_{i=1}^{I} \hat{V}_i^{y_{i*}} \right].$$

So $(U, T)$ is sufficient for $(p, \tau)$, and the joint probability mass function is

$$f(u, t; p, \tau) = \frac{t_{*}!}{u!(t_{*} - u)!} p^u (1 - p)^{t_{*} - u} \times \frac{h(t; \tau)}{\prod_{i=1}^{I} t_i!} = g_1(u; p | t) \cdot g_2(t; \tau)$$

(4)

where $g_1, g_2$ are the probability mass function of $U$ conditional on $T = t$, and the probability mass function of $T$, respectively. The derivation of the joint probability mass function is the same as in Theorem 2.1 but with $V_i \tau$ replaced by $\tilde{V}_i = V_i \tau_i$.

**Theorem 3.1.** The family $\mathcal{P}^T$ of distributions $P^T$ obtained from the joint distribution (4) is boundedly complete. Hence $T$ is complete and sufficient.

**Proof:** See Appendix.

Since $T$ is a complete and sufficient statistic, we will show that the standard binomial test is UMPU. Let $\mathcal{P}^{U,T}$ denote the family \(\{P_{p,T}^{\tau} : (p, \tau) \in \omega\}\) of distributions of $(U, T)$ with the probability mass function (4) as $(p, \tau)$ ranges over $\omega = \{(p, \tau) : p = p_0, \tau > 0\}$.

Consider the class of tests $\phi$, which satisfy

$$E_{p_0, \tau} \phi(U, T) = \alpha$$

(5)

where the expectation is with respect to $\mathcal{P}^{U,T}$. The tests (5) are similar with respect to $\mathcal{P}^{U,T}$ or $\omega$. Since the family $\mathcal{P}^T$ of distributions $P^T$ is boundedly complete, any test satisfying

$$E_{p_0, \tau} [\phi(U, T) | t] = \alpha \quad \text{a.e. } \mathcal{P}^T$$

is similar with respect to $\mathcal{P}^{U,T}$ (Lehmann (1986)). That is, these tests have Neyman Structure with respect to $T$. The next lemma shows that the most powerful test, conditional on $T = t$, is most powerful among similar tests. This lemma is Lemma 8.2 of Lehmann and Scheffé (1955).
Lemma 3.1. Let $\theta_0 \in \Theta_0 = \{(p, \tau) : p = p_0, \tau > 0\}$, $\theta_1 \in \Theta_1 = \{(p, \tau) : p > p_0, \tau > 0\}$ and so $\Theta_j \in \Theta$, $j = 0, 1$. Assume that $(\xi_1, \ldots, \xi_m)$ defined below in (7) is an inner point of the set $M$ of points in $m$-dimensional space whose coordinates are

$$(E_{\theta_0}[a_1(T)b_1(U, T)\phi(U, T)], \ldots, E_{\theta_0}[a_m(T)b_m(U, T)\phi(U, T))]$$

for some critical function $\phi$ and Borel measurable functions $a_i(t) \geq 0$ and $b_i(u, t) \geq 0$. If there exists a critical function $\phi^*(u, t)$ such that

$$E_{\theta_0}[b_i(U, T)\phi^*(U, T)|t] = \alpha_i \text{ a.e. } P^T \quad (i = 1, \ldots, m)$$

and

$$\phi^*(u, t) = \begin{cases} 
1 \text{ where } f_{\theta_1}(u, t) > \sum_{i=1}^m a_i(t)b_i(u, t)f_{\theta_0}(u, t) \\
0 \text{ where } f_{\theta_1}(u, t) < \sum_{i=1}^m a_i(t)b_i(u, t)f_{\theta_0}(u, t)
\end{cases}$$

with

$$\xi_i = E_{\theta_0}[a_i(T)b_i(U, T)\phi^*(U, T)], \quad (i = 1, \ldots, m)$$

then, $\phi^*(u, t)$ maximizes $E_{\theta_0}[\phi(U, T)]$ among all critical functions $\phi(u, t)$ which satisfy (6).

Using above lemmas, we derive a UMPU test for $H_0 : \mu_1 \leq \mu_2$ versus $H_1 : \mu_1 > \mu_2$.

3.1 A UMPU test for $H_0 : \mu_1 \leq \mu_2$ versus $H_1 : \mu_1 > \mu_2$

Theorem 3.2. The UMPU test for $H_0 : \mu_1 \leq \mu_2$ vs $H_1 : \mu_1 > \mu_2$ (equivalently, $H_0 : p \leq p_0 = 0.5$ vs $H_1 : p > p_0 = 0.5$) is given by

$$\phi(u, t) = \begin{cases} 
1 \text{ when } u > C(t) \\
\gamma(t) \text{ when } u = C(t) \\
0 \text{ when } u < C(t)
\end{cases}$$

where $C(t)$ and $\gamma(t)$ are determined by

$$E_{p_0, \tau}[\phi(U, T)|t] = \alpha \text{ a.e. } P^T \quad \text{for all } \tau > 0.$$

Proof: We will first find the most powerful test for testing $H_0 : \theta = \theta_0$ vs $H_1 : \theta = \theta_1$ where $\theta = (p, \tau)$, $\theta_0 = (p_0, \tau_0) \in \Theta_0 = \{(p, \tau) : p = p_0, \tau > 0\}$ and $\theta_1 = (p_1, \tau_1) \in \Theta_1 = \{(p, \tau) : p = p_1 > p_0, \tau > 0\}$. By Theorem 3.1, the family $P^T$ is boundedly complete, and hence similar tests $\phi(u, t)$ satisfy

$$E_{\theta}[\phi(U, T)|t] = \alpha \text{ a.e. } P^T \quad \text{for } \theta \in \Theta_0.$$

Using Lemma 3.1 with $m = 1$ and $b_1(u, t) \equiv 1$, we conclude that the most powerful test is given by

$$\phi(u, t) = \begin{cases} 
1 \text{ where } f_{\theta_1}(u, t) > a_1(t)f_{\theta_0}(u, t) \\
0 \text{ where } f_{\theta_1}(u, t) < a_1(t)f_{\theta_0}(u, t)
\end{cases}$$
Equivalently, using (4),
\[
\phi(u, t) = \begin{cases} 
1 & \text{when } g_1(u; p_1|t)/g_1(u; p_0|t) > k_1 \\
\gamma & \text{when } g_1(u; p_1|t)/g_1(u; p_0|t) = k_1 \\
0 & \text{when } g_1(u; p_1|t)/g_1(u; p_0|t) < k_1
\end{cases}
\]
where \(k_1\) may depend on \((t, \tau_0, \tau_1)\). Since \(g_1(u; p|t)\) is the probability mass function of the binomial distribution with parameters \(p\) and \(u\), the conditional likelihood ratio \(g_1(u; p_1|t)/g_1(u; p_0|t)\) is a nondecreasing function of \(u\). Therefore, \(\phi\) is such that
\[
\phi(u, t) = \begin{cases} 
1 & \text{when } u > k_1^* \\
\gamma & \text{when } u = k_1^* \\
0 & \text{when } u < k_1^*
\end{cases}
\]
where \(k_1^*\) may depend on \((t, p_0, p_1, \tau_0, \tau_1)\). Since the distribution of \(U\) conditional on \(T = t\) does not depend on \((t, \tau_0, \tau_1)\), we can choose \(k_1^*\) and \(\gamma\) independently of \((t, \tau_0, \tau_1)\), and hence (10) is equivalent to (9). The test (11) is also free of \(p_1\) for \(p_1 > p_0\). Thus the UMP unbiased test is given by (8) and (9) provided that \(\xi = \alpha \cdot E_{p_0, \tau}[a_1(T)]\) is an inner point of \(M\) where \(M\) is the set of points \(E_{p_0, \tau}[a_1(T)|\phi(u, T)]\) as \(\phi\) ranges over the totality of critical functions. But \(M\) is convex and closed. Also, taking \(\phi = \delta\), \(M\) contains all points \(\delta \cdot E_{p_0, \tau}[a_1(T)]\) with \(0 < \delta < 1\), so \(\xi\) is an inner point of \(M\).

Further, the measurability of \(\phi(U, T)\) is obvious since the distribution of \(U\) conditional on \(T = t\) is the binomial distribution.

\[\Box\]

3.2 A UMPU test for \(H_0 : \mu_1 = \mu_2\) versus \(H_1 : \mu_1 \neq \mu_2\)

In this subsection we derive a UMPU test for \(H_0 : \mu_1 = \mu_2\) versus \(H_1 : \mu_1 \neq \mu_2\) using the following lemma (Lehmann (1986), page 117).

Lemma 3.2. For the UMPU test \(\phi\) defined in (8) and (9),
\[
E_{p, \tau}[a(T) \cdot U\phi(U, T)] - E_{p, \tau}[a(T) \cdot pI_\phi(U, T)] > 0 \quad \text{for all } 0 < p < 1, \tau > 0.
\]
where \(a(T)\) is any nonnegative Borel measurable function.

Theorem 3.3. The UMPU test for \(H_0 : \mu_1 = \mu_2\) versus \(H_1 : \mu_1 \neq \mu_2\) (equivalently, \(H_0 : p = p_0 = 0.5\) versus \(H_1 : p \neq p_0 = 0.5\)) is given by
\[
\phi(u, t) = \begin{cases} 
1 & \text{when } u < C_1(t) \text{ or } u > C_2(t) \\
\gamma_i(t) & \text{when } u = C_i(t), \quad i = 1, 2 \\
0 & \text{when } C_1(t) < u < C_2(t)
\end{cases}
\]

(12)
where the $C$'s and $\gamma$'s are determined by
\begin{equation}
E_{p_0, \tau}[\phi(U, T)|t] = \alpha \quad \text{a.e. } P^T \quad \text{for all } \tau > 0.
\end{equation}

and
\begin{equation}
E_{p_0, \tau}[U \phi(U, T)|t] = \alpha \cdot p_0 t_\ast \quad \text{a.e. } P^T \quad \text{for all } \tau > 0.
\end{equation}

**Proof:** Consider the power function $\beta(p, \tau) = E_{p, \tau}[\phi(U, T)]$. Under $H_0 : \theta = \theta_0$,
\[ \beta(p_0, \tau_0) = E_{p_0, \tau_0}[\phi(U, T)] = \alpha \quad \text{for all } \tau_0 > 0. \]

By the boundedly completeness of $T$ established in Theorem 3.1, this implies that
\begin{equation}
\beta(p_0, \tau_0) = E_{p_0, \tau_0}[\phi(U, T)|t] = \alpha \quad \text{a.e. } P^T \quad \text{for all } \tau_0 > 0.
\end{equation}

Because the test is unbiased, its power function $\beta(p, \tau_0)$ must have a minimum at $p = p_0$. Hence,
\begin{equation}
\frac{\partial}{\partial p} \beta(p, \tau_0)|_{p=p_0} = 0 \quad \text{for all } \tau_0 > 0.
\end{equation}

Since
\begin{align*}
\frac{\partial}{\partial p} \beta(p, \tau_0) &= \frac{\partial}{\partial p} \sum_{t,u} \phi(u, t) \left( \frac{t_\ast}{u} \right) p^u (1-p)^{t_\ast-u} g_2(t; \tau_0) \\
&= \sum_{t,u} \left\{ \frac{u}{p} - \frac{t_\ast - u}{1-p} \right\} \phi(u, t) \left( \frac{t_\ast}{u} \right) p^u (1-p)^{t_\ast-u} g_2(t; \tau_0) \\
&= \frac{1}{p(1-p)} E_{p, \tau_0}[(U - p T_\ast) \phi(U, T)],
\end{align*}

The condition (16) implies that
\[ E_{p_0, \tau_0}[U \phi(U, T)] = \alpha \cdot E_{p_0, \tau_0}[p_0 T_\ast] \quad \text{for all } \tau_0 > 0. \]

By boundedly completeness, this reduces to
\begin{equation}
E_{p_0, \tau_0}[U \phi(U, T)|t] = \alpha \cdot p_0 t_\ast \quad \text{a.e. } P^T \quad \text{for all } \tau_0 > 0.
\end{equation}

Using Lemma 3.1 with (15) and (17), or $m = 2$, $b_1(u, t) = 1$, $b_2(u, t) = u$, $\alpha_1 = \alpha$ and $\alpha_2 = \alpha \cdot p_0 t_\ast$, we conclude that any $\phi$ that maximizes $E_{\theta, 1}[\phi(u, t)]$ is given by
\[ \phi(u, t) = \begin{cases} 
1 & \text{where } f_{\theta_1}(u, t) > \{a_1(t) + u \cdot a_2(t)\} f_{\theta_0}(u, t) \\
0 & \text{where } f_{\theta_1}(u, t) < \{a_1(t) + u \cdot a_2(t)\} f_{\theta_0}(u, t)
\end{cases} \]
Equivalently, by (4),

\[
\phi(u, t) = \begin{cases} 
1 & \text{where } g_1(u; p_1|t)/g_1(u; p_0|t) > k_1 + u \cdot k_2 \\
0 & \text{where } g_1(u; p_1|t)/g_1(u; p_0|t) < k_1 + u \cdot k_2 
\end{cases}
\]

where \( k_1 \) may depend on \((t, \tau_0, \tau_1)\). Further, since \( g_1(u; p|t) \) is binomial,

\[
\phi(u, t) = \begin{cases} 
1 & \text{where } k_1^* + k_2^* \cdot u < \exp[k_3^* \cdot u] \\
\gamma & \text{where } k_1^* + k_2^* \cdot u = \exp[k_3^* \cdot u] \\
0 & \text{where } k_1^* + k_2^* \cdot u > \exp[k_3^* \cdot u] 
\end{cases}
\]

where the \( k_j^* \)'s may depend on \((t, p_0, p_1, \tau_0, \tau_1)\). However, neither the null nor the alternative conditional distribution of \( U \) given \( T = t \) depends on \((t, \tau_0, \tau_1)\) and hence we can choose the \( k_j^* \)'s to be free of \((t, \tau_0, \tau_1)\). As in Lehmann (1986), page 103, the region \( k_1^* + k_2^* \cdot u < \exp[k_3^* \cdot u] \) is either one-sided or the outside of an interval. It is well known that the one-sided test has a strictly monotone power function for the binomial distribution and therefore cannot satisfy the condition (14). Thus \( \phi \) is 1 when \( u < C_1(t) \) or \( > C_2(t) \), and the most powerful test subject to (13), (14) is given by (12). Since the class of tests satisfying (12), (13) and (14) includes the class of unbiased tests, this test is unbiased and also UMPU whenever \((\xi_1, \xi_2) = (\alpha E_{p_0, T}[a_1(T)], \alpha \cdot E_{p_0, T}[a_2(T) \cdot p_0 T^*])\) is an inner point of \( M \) where \( M \) is the set of points \((E_{p_0, \tau_0}[a_1(T)] \phi(U, T)), E_{p_0, \tau_0}[a_2(T) \cdot U \phi(U, T)]\) as \( \phi \) ranges over the totality of critical functions. But \( M \) is convex and closed. Using (15) and (17), and replacing \( \alpha \) by \( \delta \), \( M \) contains all points \((\delta E_{p_0, T}[a_1(T)], \delta \cdot E_{p_0, T}[a_2(T) \cdot p_0 T^*])\) with \( 0 < \delta < 1 \). By Lemma 3.2, it also contains points \((\alpha E_{p_0, T}[a_1(T)], \delta_1)\) with \( \delta_1 > \alpha \cdot E_{p_0, T}[a_2(T) \cdot p_0 T^*] \). Similarly, \( M \) contains points \((\alpha E_{p_0, T}[a_1(T)], \delta_2)\) with \( \delta_2 < \alpha \cdot E_{p_0, T}[a_2(T) \cdot p_0 T^*] \). Hence \((\xi_1, \xi_2)\) is an inner point of \( M \).

Further, as in the previous theorem, the measurability of \( \phi(U, T) \) is obvious since the distribution of \( U \) conditional on \( T = t \) is binomial.

4 Comparisons of unconditional powers

4.1 Setup and assumptions

In this section, we evaluate the unconditional powers of the proposed conditional test, the binomial test, and the appropriate a likelihood ratio (LR) test. We take the unconditional expectation of the sum, \( \mu_\ast = \mu_1 + \mu_2 \), to be either 10 or 50 and study the power of the tests for alternatives having \( p = \mu_1/(\mu_1 + \mu_2) = 0.50, 0.51, \ldots, 0.99 \). The sample sizes, \( I \), are chosen as 1 or 5. Powers of tests are calculated numerically, but a simulation is used to calculate the power of tests when \( I = 5 \) (based on 10,000 repetitions of the test under the same alternative hypothesis). Two distributions, the gamma and inverse Gaussian were
selected for the random effects. Also we set the mean of random effects equal to 1 and that distribution with a standard deviation, \( \sigma \), equal to 1, 2.5 and 5.

For the conditional test, when the sum of both counts is equal to zero, we randomize and reject the null hypothesis \( H_0 : p = 0.5 (\mu_1 = \mu_2) \) with a probability of 0.05 to have a significance level of 0.05.

For the LR test, we make some reasonable assumptions: (i) when one of the counts is zero, we choose the estimate of the parameter equal to the boundary value, (ii) when both counts are zero, we arbitrarily set a test statistic equal to zero so that a null hypothesis is accepted in our calculation. Since the LR is continuous, we can choose the estimate very close to zero, and setting it equal to zero does not affect our result.

Hence the LR test is,

\[
-2 \log(LR) = \begin{cases} 
2 \left( y_{a1} \log \frac{2y_{a1}}{y_{**}} + y_{a2} \log \frac{2y_{a2}}{y_{**}} \right) & \text{when } y_{a1} \neq 0, y_{a2} \neq 0. \\
2(y_{a} - y_{a}) \log 2 & \text{when } y_{a} = 0 \ (j = 1, 2).
\end{cases}
\]

In all cases, the large sample \( \chi^2 \) critical value was used.

### 4.2 Study of unconditional power

Figure (a) shows the effect of the standard deviation of random effects on the power of the tests with \( I = 1 \) and \( \mu_* = 10 \). The random effects follow a gamma distribution with mean 1 and \( \sigma = 1, 2.5 \) and 5. The LR test, overall, is unstable and failed to control the probability of type I error. This is because the \( \chi^2 \) approximation to the LR test fails for a small sample size. The conditional test is more stable and has higher power than the LR test as the the standard deviation of random effect increases.

Figure (b) shows the effect of sample size when \( \mu_* = 10 \) and \( \sigma = 5 \) (the power curve for \( I = 5 \) is obtained by simulation). The power curves of both tests are greatly improved and similar.

Figure (c) shows the effect of the size of \( \mu_* \) on the power where \( I = 1 \) and \( \sigma = 5 \). Increasing \( \mu_* \) increases the power, but it does not improve as much as the sample size does. The LR test tends to have the lower power and cannot control the probability of type I error.

Figure (d) shows that the power curves using different random effects distribution with \( I = 1, \mu_* = 10, \sigma = 5 \). Powers are generally higher for inverse Gaussian distribution, but note that the conditional test controls the probability of type I error regardless of the distribution of random effects, while the level of the LR test is not controlled over all choices of the random effects. Using the inverse Gaussian distribution, with the same mean and the variance, gave similar results and are omitted.

Omori (1992) performed an extensive simulation for enriched random effects model where \( \tau_1, \tau_2, \ldots, \tau_I \) are not equal. As we increase the standard deviation of the \( \tau_i \)'s, \( s_\tau \), the power goes down overall. Since we fixed the sum of \( \tau_i \)'s in the cases considered, an increase in \( s_\tau \) implies more small \( \tau \)'s and less large \( \tau \)'s. When heterogeneous data are mixed together, it is natural to expect lower power. The LR test tended to have higher power than the
conditional tests, but it failed to control the probability of type I error. It was our aim to develop a test (12)-(14) having a good power that controlled the type I error probability.

Appendix

Proof of Theorem 2.1  The characteristic function of $\mathbf{Y} = (Y_{11}, \ldots, Y_{I2})$ is

$$\varphi(t) = E[exp\{i \sum_{i=1}^{I} \sum_{j=1}^{2} t_{ij}Y_{ij}\}] = E_{V}[exp\{\tau \sum_{i=1}^{I} \sum_{j=1}^{2} V_{ij}p_{j}(e^{it_{ij}} - 1)\}].$$

(18)

First, the probability mass functions

$$g_{3}(y_{**}; \tau) = \frac{\tau^{y_{**}}E_{V}[e^{-\tau \sum_{i=1}^{I} V_{i}(\sum_{i=1}^{I} V_{i})^{y_{**}}}]}{y_{**}!},$$

$$g_{1}(y_{*1}; p|y_{**})g_{3}(y_{**}; \tau) = \frac{y_{**}!}{\prod_{j=1}^{2} y_{*j}!} \prod_{j=1}^{2} p_{j}^{y_{*j}} \times g_{3}(y_{**}; \tau),$$

for $Y_{**}, (Y_{*1}, Y_{**})$ have the characteristic functions (18) with $t_{ij} = t$ and $t_{ij} = t + s_{j}, (i = 1, \ldots, I, j = 1, 2), s_{2} = 0$ respectively. Also the probability mass functions

$$g_{2}(y_{1*}, \ldots, y_{I-1*}; \tau|y_{**})g_{3}(y_{**}; \tau) = \frac{E_{V}[e^{-\tau \sum_{i=1}^{I} V_{i}(\sum_{i=1}^{I} V_{i})^{y_{**}}}]}{E_{V}[e^{-\tau \sum_{i=1}^{I} V_{i}(\sum_{i=1}^{I} V_{i})^{y_{**}}}]} \times g_{3}(y_{**}; \tau),$$

and $g_{1}(y_{*1}; p|y_{**})g_{2}(y_{1*}, \ldots, y_{I-1*}; \tau|y_{**})g_{3}(y_{**}; \tau)$ for $(Y_{1*}, \ldots, Y_{I-1*}, Y_{**}), (Y_{1*}, \ldots, Y_{I*}, Y_{1})$ have the characteristic function (18) with $t_{ij} = t + s_{j}, (i = 1, \ldots, I, j = 1, 2), t_{I} = 0,$ and $t_{ij} = t_{i} + s_{j} (i = 1, \ldots, I, j = 1, 2), s_{2} = 0$ respectively.

Finally, for (3), the probability mass function

$$g_{2}(y_{1*}, \ldots, y_{I*}; \tau) = \frac{\tau^{y_{**}}E_{V}[e^{-\tau \sum_{i=1}^{I} V_{i}(\sum_{i=1}^{I} V_{i})^{y_{**}}}]}{\prod_{i=1}^{I} y_{i*}!},$$

for $(Y_{1*}, \ldots, Y_{I*}),$ has the characteristic function (18) with $t_{ij} = t_{i} (i = 1, \ldots, I, j = 1, 2).$

Proof of Lemma 2.1.

$$c_{\psi}(\omega) = \int \psi(y)e^{-i\omega'y}dy = \int \int f(y-x)e^{-i\omega'(y-x)}g(x)e^{-i\omega'x}dydx = \int f(z)e^{-i\omega'z}dz \int g(x)e^{-i\omega'x}dx = c_{f}(\omega)c_{g}(\omega).$$
Proof of Lemma 2.2. Consider the bounded function $b(x)$ such that $E(b(X)) = 0$. This implies

$$0 = \int b(x)f(x-\eta)dx = \int f^*(\eta-x)b(x)dx \equiv \psi(\eta), \quad f^*(\eta-x) = f(x-\eta),$$

which is a convolution of $f^*$ and $b$. Then, since $b(x)$, $f(x)$ are absolutely integrable on $\mathbb{R}$, the Fourier transformation of the convolution is, by Lemma 2.1,

$$0 = c_\psi(\omega) = c_{f^*}(\omega)c_b(\omega),$$

where $\omega = (\omega_1, \ldots, \omega_I)$, $\{\omega_j : -\infty < \omega_j < \infty\}$, $j = 1, \ldots, I$ and $c_{f^*}(\omega)$, $c_b(\omega)$ are the Fourier transformation of $f^*$, $b$ respectively. If the characteristic function of $f^*$ is not equal to zero for any $t$, $c_{f^*}(\omega) \neq 0$, for all $\omega$, and hence $c_b(\omega) = 0$, for all $\omega$. Therefore, by the uniqueness of the Fourier transformation, $b(x) \equiv 0$, and the location family is boundedly complete. On the other hand, if the characteristic function of $f^*$ is equal to zero for some $t$, then $c_{f^*}(\omega) = 0$, for some $\omega$, and hence there exists $b(x)$ satisfying $c_b(\omega) \neq 0$ for some $\omega$. Therefore, $b(x)$ is not identically zero, and the location family is not boundedly complete.

\[\square\]

Proof of Theorem 2.2. (i) From (2), $P_{T^*}^T$ has the probability mass function

$$P(T_\bullet = t_\bullet) = \frac{H(t_\bullet, \tau)}{t_\bullet!} \quad \text{for} \quad t_\bullet = 0, 1, 2 \ldots$$

where $H(t_\bullet, \tau) = \tau^k E_W[e^{-W\tau}W^k]$ and $W = \sum_{i=1}^I V_i$. Let $b(t_\bullet)$ be any bounded function such that $E[b(T_\bullet)] = 0$ for all $P_{T^*}^T \in \mathcal{P}_{T^*}$.

Then, setting $Z = \tau W$,

$$0 = \sum_{t_\bullet=0}^\infty b(t_\bullet) \frac{H(t_\bullet, \tau)}{t_\bullet!} = \sum_{k=0}^\infty b(k) \frac{\tau^k E_W[e^{-W\tau}W^k]}{k!} = E_Z \left[ \sum_{k=0}^\infty b(k) \frac{e^{-z}z^k}{k!} \right]$$

By Lemma 2.2 with $\eta = -\log \tau$, $X = \log Z$, $\mathcal{P}^Z$ is boundedly complete and hence the integrand vanishes for almost all $z$. Because

$$\sum_{k=0}^\infty \frac{b(k)}{k!} z^k = 0 \quad \text{for some} \quad z > 0,$$

and the series is absolutely convergent, all the coefficients of which must be zero. Hence,

$$b(t_\bullet) = 0 \quad \text{for} \quad t_\bullet = 0, 1, 2 \ldots$$

and hence the family $\mathcal{P}_{T^*}$ of distributions $P_{T^*}^T$ for $T_\bullet$ is boundedly complete. The statistic $T_\bullet$ is not sufficient since the distribution of $T$ given $T_\bullet = t_\bullet$ is dependent of $\tau$. 

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(ii) For the family of distributions of $T$, consider $g(t) = e^{-t}$. Since $0 \leq g(t) \leq 1$ for all $t \geq 0$, $E(g(T))$ exists. Now let $b(t) = g(t_1) - g(t_2)$. Then $E(b(T)) = E(g(T')) - E(g(T)) = 0$. Therefore, the family of distributions of $T$ is not boundedly complete. On the other hand, the statistic $T$ is obviously sufficient. Hence, the statistic $T$ is sufficient but not boundedly complete.

Proof of Theorem 3.1. Consider the bounded function $b(t)$ such that $E(b(t)) = 0$. Then,

$$\int \left[ \sum_{t} b(t) \prod_{i=1}^{I} \frac{e^{-\tilde{\nu}_{i} t_{i}^{t_{i}}}}{t_{i}!} \right] g(\tilde{\nu}_{1}/\tau_{1}, \ldots, \tilde{\nu}_{I}/\tau_{I}) \prod_{i=1}^{I} \frac{d\tilde{\nu}_{i}}{\tau_{i}} = 0,$$

for all $\tau_{1}, \ldots, \tau_{I} > 0$, where $g$ is a probability density function such that the characteristic function of $X = (\log \tilde{V}_{1}, \ldots, \log \tilde{V}_{I})'$, $\varphi(s)$, is not equal to zero for any value of $s$. By Lemma 2.2 with $\eta_{t} = -\log \tau_{t}$, $X_{t} = \log \tilde{V}_{t}$ ($i = 1, 2, \ldots, I$), the family of distributions associated with $(\tilde{V}_{1}, \ldots, \tilde{V}_{I})$ is boundedly complete, and hence

$$\sum_{t} b(t) \prod_{i=1}^{I} \frac{e^{-\tilde{\nu}_{i} t_{i}^{t_{i}}}}{t_{i}!} \equiv 0.$$

almost every $(\tilde{\nu}_{1}, \ldots, \tilde{\nu}_{I})'$. By continuity, and since the multivariate power series is absolutely convergent, $b(t) \equiv 0$, and the result follows.

$\square$
References


Figure 1: Unconditional Power of tests

---: conditional test, - - - : LR test.
Horizontal dashed line corresponds to the level of 0.05.
(a): $\mu_* = 10, I = 1$, gamma distribution for random effects.
(b): $\mu_* = 10, \sigma = 5$, gamma distribution for random effects.
(c): $I = 1, \sigma = 5$, gamma distribution for random effects.
(d): $\mu_* = 10, I = 1, \sigma = 5$. 