

# **STAT 426**

Author: Wenxiao Yang

Institute: Department of Mathematics, University of Illinois at Urbana-Champaign

All models are wrong, but some are useful.

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## **Chapter 1 Basic of Categorical Data**

#### 1.1 Variable Measurement

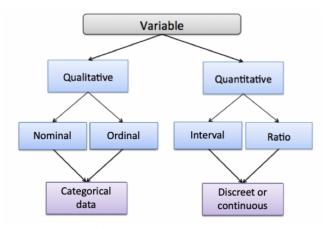


Figure 1.1: Variable Type

- a) Nominal: Categories do not have a natural order. Ex. blood type, gender.
- b) Ordinal: Categories have a natural order. Ex. low/middle/high education level
- c) Interval: There is a numerical distance (difference between two different values is meaningful) between any two values. Ex. blood pressure level, 100 blood pressure doesn't mean the double degree of 50 pressure.
- d) Ratio: An interval variable where ratios are valid (presence of absolute zero, i.e. zero is meaningful). Ex. weight, 4g is double degree of 2g, distance run by an athlete.

#### Levels of measurements

A variable's level of measurement determines the statistical methods to be used for its analysis.

Variables hierarchy: Ratio > Interval > Ordinal > Nominal

Statistical methods applied to variables at a lower level can be used with variables at a higher level, but the contrary is not true.

## 1.2 Statistical Inference for Categorical Data

There is a distribution  $F(\beta)$  with p.d.f. (p.m.f.)  $f(x \mid \beta)$ , where  $\beta$  a generic unknown parameter and  $\hat{\beta}$  the parameter estimate.

#### 1.2.1 Maximum likelihood Estimation (MLE)

Given a set of observations  $\vec{x} = (x_1, ..., x_n)$ , the likelihood function of these observations with parameter  $\beta$  is  $l(\vec{x} \mid \beta)$ . We want to find parameter  $\hat{\beta}$  that maximizes the likelihood function,

$$\hat{\beta} = \arg\max_{\beta} l(\vec{x} \mid \beta)$$

which is also equivalent to maximizing the logarithm of the likelihood function  $L(\vec{x} \mid \beta) = \log(l(\vec{x} \mid \beta))$ ,

$$\hat{\beta} = \arg\max_{\beta} L(\vec{x} \mid \beta)$$

#### **Definition 1.1 (score function)**

The score function is

$$u(\beta, \vec{x}) = \nabla_{\beta} L(\vec{x} \mid \beta) = \frac{\nabla_{\beta} l(\vec{x} \mid \beta)}{l(\vec{x} \mid \beta)}$$

#### **Lemma 1.1 (mean of score function)**

*The mean of score function is* 0,

$$\mathbb{E}_{\vec{x}}u(\beta,\vec{x}) = 0$$

Proof 1.1

$$\mathbb{E}_{\vec{x}}u(\beta, \vec{x}) = \int_{\vec{x}} l(\vec{x} \mid \beta) \frac{\nabla_{\beta} l(\vec{x} \mid \beta)}{l(\vec{x} \mid \beta)} d\vec{x}$$
$$= \int_{\vec{x}} \nabla_{\beta} l(\vec{x} \mid \beta) d\vec{x}$$
$$= \nabla_{\beta} \left( \int_{\vec{x}} l(\vec{x} \mid \beta) d\vec{x} \right)$$
$$= \nabla_{\beta} 1 = 0$$

#### **Lemma 1.2 (variance of score function)**

The variance of the score function is

$$\operatorname{Var}_{\vec{x}}(u(\beta, \vec{x})) = \mathbb{E}_{\vec{x}}\left(u(\beta, \vec{x})u(\beta, \vec{x})^{T}\right)$$

#### Proof 1.2

Prove by the zero mean.

#### **Definition 1.2 (Fisher information)**

The (Fisher) information is

$$\iota(\beta) = -\mathbb{E}_{\vec{x}} \left[ \nabla_{\beta}^2 L(\vec{x} \mid \beta) \right]$$

#### Lemma 1.3

The Fisher information is equal to the variance of score function.

$$\operatorname{Var}_{\vec{x}}(u(\beta, \vec{x})) = \mathbb{E}_{\vec{x}}\left(u(\beta, \vec{x})u(\beta, \vec{x})^T\right) = -\mathbb{E}_{\vec{x}}\left[\nabla_{\beta}^2 L(\vec{x} \mid \beta)\right] = \iota(\beta)$$

#### Proof 1.3

$$\mathbb{E}_{\vec{x}} \left[ \nabla_{\beta}^2 L(\vec{x} \mid \beta) \right] = \mathbb{E}_{\vec{x}} \left( \frac{\partial \frac{\nabla_{\beta} l(\vec{x} \mid \beta)}{l(\vec{x} \mid \beta)}}{\partial \beta} \right) = \mathbb{E}_{\vec{x}} \left( \frac{\nabla_{\beta}^2 l(\vec{x} \mid \beta)}{l(\vec{x} \mid \beta)} - \frac{\nabla_{\beta} l(\vec{x} \mid \beta) \nabla_{\beta} l(\vec{x} \mid \beta)^T}{(l(\vec{x} \mid \beta))^2} \right)$$

where 
$$\mathbb{E}_{\vec{x}}\left(\frac{\nabla_{\beta}^2 l(\vec{x}|\beta)}{l(\vec{x}|\beta)}\right) = \int_{\vec{x}} l(\vec{x} \mid \beta) \frac{\nabla_{\beta}^2 l(\vec{x}|\beta)}{l(\vec{x}|\beta)} d\vec{x} = \int_{\vec{x}} \nabla_{\beta}^2 l(\vec{x} \mid \beta) d\vec{x} = \nabla_{\beta}^2 \int_{\vec{x}} l(\vec{x} \mid \beta) d\vec{x} = \nabla_{\beta}^2 1 = 0$$
Hence.

$$\mathbb{E}_{\vec{x}}\left[\nabla_{\beta}^{2}L(\vec{x}\mid\beta)\right] = -\mathbb{E}_{\vec{x}}\left(\frac{\nabla_{\beta}l(\vec{x}\mid\beta)\nabla_{\beta}l(\vec{x}\mid\beta)^{T}}{(l(\vec{x}\mid\beta))^{2}}\right) = -\mathbb{E}_{\vec{x}}\left(u(\beta,\vec{x})u(\beta,\vec{x})^{T}\right)$$

#### **Proposition 1.1**

When the sample x is made up of i.i.d. observations, the covariance matrix of the maximum likelihood estimator  $\hat{\beta}$  is approximately equal to the inverse of the information matrix.

$$Cov(\hat{\beta}) \approx (\iota(\beta))^{-1}$$

Hence, the covariance matrix can be estimated as  $(\iota(\hat{\beta}))^{-1}$ . Similarly, SE is estimated by  $\sqrt{(\iota(\hat{\beta}))^{-1}}$ .

#### 1.2.2 Likelihood Inference (Wald, Likelihood-Ratio, Score)

We want to test

$$H_0: \beta = \beta_0 \qquad H_\alpha: \beta \neq \beta_0$$

or form a confidence interval (CI) for  $\beta$ .

#### **Definition 1.3 (Wald Test)**

The Wald statistic:

$$z_W = \frac{\hat{\beta} - \beta_0}{SE} = \frac{\hat{\beta} - \beta_0}{\sqrt{(\iota(\hat{\beta}))^{-1}}}$$

where 
$$SE = \sqrt{(\iota(\hat{\beta}))^{-1}}$$
.

Usually, as  $n \to \infty$ ,  $z_W \xrightarrow{d} N(0,1)$  under  $H_0: \beta = \beta_0$ .

(1) We reject the  $H_0$  if  $|z_W| \ge z_{\frac{\alpha}{2}}$  for a two-sided level  $\alpha$  test.

(2) The  $(1-\alpha)100\%$  Wald (confidence) interval is

$$\{\beta_0 : |z_W| = \frac{|\hat{\beta} - \beta_0|}{SE} < z_{\frac{\alpha}{2}}\} = (\hat{\beta} - z_{\frac{\alpha}{2}}SE, \hat{\beta} + z_{\frac{\alpha}{2}}SE)$$

(3) The Wald test also has a chi-squared form, using

$$z_W^2 = \frac{(\hat{\beta} - \beta_0)^2}{(\iota(\hat{\beta}))^{-1}} \sim \chi_1^2 \quad \text{(under } H_0\text{)}$$

#### **Definition 1.4 (Likelihood Ratio Test)**

Let

$$\Lambda = \frac{l(\vec{x} \mid \beta_0)}{l(\vec{x} \mid \hat{\beta})}$$

where  $l(\vec{x} \mid \hat{\beta}) = \max_{\beta} l(\vec{x} \mid \beta)$ , so the ratio  $\Lambda \in [0, 1]$ .

The likelihood-ratio test (LRT) chi-squared statistic:

$$-2\ln\Lambda = -2\left(L(\beta_0) - L(\hat{\beta})\right)$$

It has an approximate  $\chi_1^2$  distribution under  $H_0: \beta = \beta_0$ , and otherwise tends to be larger.

(1) Thus, reject  $H_0$  if

$$-2\ln\Lambda \ge \chi_1^2(\alpha)$$

(2) The  $(1-\alpha)100\%$  likelihood-ratio (confidence) interval is

$$\{\beta_0: -2\ln\Lambda = -2\left(L(\beta_0) - L(\hat{\beta})\right) < \chi_1^2(\alpha)\}$$

*Unlike Wald, this interval is* <u>not degenerate</u>. (i.e., For general case, the interval does not have an explicit form.)

#### **Definition 1.5 (Score Test)**

The score statistic:

$$z_S = \frac{u(\beta_0)}{\sqrt{\iota(\beta_0)}}$$

As  $n \to \infty$ ,  $z_S \stackrel{d}{\longrightarrow} N(0,1)$  under  $H_0: \beta = \beta_0$ . Otherwise, it tends to be further from zero.

- (1) Thus, reject  $H_0$  if  $|z_S| \ge z_{\frac{\alpha}{2}}$  for a <u>two-sided level  $\alpha$  test</u>.
- (2) The  $(1-\alpha)100\%$  score (confidence) interval is

$$\{\beta_0 : |z_S| = \frac{|u(\beta_0)|}{\sqrt{\iota(\beta_0)}} < z_{\frac{\alpha}{2}}\}$$

Unlike Wald, it is not degenerate for some distributions.

(3) There is also a chi-squared form:

$$z_S^2 = rac{u(eta_0)^2}{\iota(eta_0)} \sim \chi_1^2 \quad ext{(under } H_0)$$

We can also use P-value to measure the probability of the statistic is more extreme under the  $H_0$ . We can reject  $H_0$  if the P-value is  $\leq \alpha$ .

All three kinds tend to be "asymptotically equivalent" as  $n \to \infty$ . For smaller n, the <u>likelihood-ratio</u> and <u>score</u> methods are preferred.

## **Chapter 2 Association in Contingency Tables**

## 2.1 Association in Two-Way Contingency Tables

Consider joint observations of two categorical variables: X with I categories, Y with J categories.

We can summarize data in an  $I \times J$  contingency table:

$$\begin{array}{c|cccc} & & & Y & \\ & & 1 & \cdots & J & \\ \hline & & & & \\ X & \vdots & & & \\ I & & & & \\ \end{array}$$

Each cell contains a count.

#### 2.1.1 Distribution

If both X and Y are random, let

$$\pi_{ij} = P(X \text{ in row } i, Y \text{ in col } j)$$

be the **joint** distribution of X and Y.

The **marginal** distribution of X is defined by

$$\pi_{i+} = P(X \text{ in row } i)$$

and similarly for Y:

$$\pi_{+j} = P(Y \text{ in col } j)$$

The **conditional** distribution of Y given that X is in row i is defined by

$$\pi_{j|i} = P(Y \text{ in col } j \mid X \text{ in row } i) = \frac{\pi_{ij}}{\pi_{i+}}$$

#### 2.1.2 Independent / Homogeneity

#### **Definition 2.1 (independent)**

If both X and Y are <u>random</u>, they are **independent** if

$$\pi_{ij} = \pi_{i+}\pi_{+j}, \ \forall i,j$$

which implies  $\pi_{j|i} = \frac{\pi_{i+}\pi_{+j}}{\pi_{i+}} = \pi_{+j}, \forall i, j$ . That is,  $\pi_{j|i}$  doesn't depend on i and is the same as the

marginal distribution of Y. (Intuitively, knowing X tells nothing about Y.)

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#### **Definition 2.2 (homogeneity)**

Even if X is <u>not really random</u>, the condition that  $\pi_{j|i} = \pi_{+j}, \forall i, j$  is called **homogeneity**. This might still be relevant in a situation where X is deliberately chosen and Y is observed as a response.

#### \*

#### 2.1.3 Descriptive Statistics

Let  $n_{ij} = count$  in row i and col j and  $n = \sum_{i} \sum_{j} n_{ij}$ .

The margins of the table:

$$n_{i+} = \sum_{j} n_{ij}, \quad n_{+j} = \sum_{i} n_{ij}$$

#### **Natural Estimation**

- 1. Natural estimate of  $\pi_{ij}$ :  $p_{ij} = \frac{n_{ij}}{n}$
- 2. Similarly marginals:  $p_{i+} = \sum_j p_{ij}$   $p_{+j} = \sum_i p_{ij}$
- 3. And conditionals:  $p_{j|i} = \frac{p_{ij}}{p_{i+}} = \frac{n_{ij}}{n_{i+}}$

#### **2.1.4 Sampling Models (Examples)**

Possible joint distributions for counts in  $I \times J$  table:

1. Poisson (random total):  $Y_{ij} = \text{count in cell } (i, j),$ 

$$Y_{ij} \sim \text{Poisson}(\mu_{ij})$$

and the  $Y_{ij}$ s are independent.

2. Multinomial (fixed total n):  $N_{ij} = \text{count in cell } (i, j)$ ,

$$\{N_{ij}\} \sim \text{multinomial}(n, \{\pi_{ij}\})$$

3. Independent Multinomial: Assume  $n_{i+}$  (row totals  $n_i$ ) are fixed,

$$\left\{ N_{1j} \right\}_{j=1}^{J} \sim \text{multinomial}(n_1, \left\{ \pi_{j|1} \right\}_{j=1}^{J})$$

$$\vdots$$

$$\left\{ N_{Ij} \right\}_{j=1}^{J} \sim \text{multinomial}(n_I, \left\{ \pi_{j|I} \right\}_{j=1}^{J})$$

(When J=2, this is independent binomial sampling, for which we may just write  $\pi_i$  for  $\pi_{1|i}$ .)

#### 2.1.5 Measuring Inhomogeneity

Homogeneity is the condition  $\pi_1 = \pi_2$ . We can measure inhomogeneity by:

1. difference of proportions:

$$\pi_1 - \pi_2$$

2. relative risk:

$$RR = \frac{\pi_1}{\pi_2}$$

3. odds ratio:

$$\theta = \frac{\pi_1/(1-\pi_1)}{\pi_2/(1-\pi_2)}$$

When  $\theta = 1$ , we can say there is no association.

The **odds** for a probability  $\pi$  is  $\Omega = \frac{\pi}{1-\pi}$ . Note  $\pi = \frac{\Omega}{1+\Omega}$ .

(In the multinomial model:  $\theta = \frac{\pi_{11}\pi_{22}}{\pi_{12}\pi_{21}}$  ("cross-product ratio"); in Poisson model:  $\theta = \frac{\mu_{11}\mu_{22}}{\mu_{12}\mu_{21}}$ )

The usual (unrestricted) estimates

$$\hat{\theta} = \frac{n_{11}n_{22}}{n_{12}n_{21}}$$

Useful properties of odds ratio:

- (1) Interchanging rows (or cols) changes  $\theta$  to  $\frac{1}{\theta}$ .
- (2) Interchanging X and Y doesn't change  $\theta$ .
- (3) Multiplying a row (or col) by a factor doesn't change  $\hat{\theta}$ .
- (4) Relationship to relative risk:  $\theta=RR\cdot\frac{1-\pi_2}{1-\pi_1}$ . ( $\theta$  and RR are similar if both  $\pi_1$  and  $\pi_2$  are small.)

## 2.2 Conditional Association in Three-Way Tables

Add a third categorical variable Z.

**Example 2.1** Is a drug more effective at curing a disease among younger patients than among older? X = drug or placebo; Y = disease cured or not; Z = age group (young, old).

#### 2.2.1 Conditional Association

Z may be called a **stratification variable**. We are interested in the distribution of (X, Y) conditional on Z.

#### **Definition 2.3 (partial table)**

Each Z category defines a partial table for X and Y.

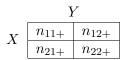
.

**Example 2.2** When Z = 1, 2 and X, Y are binary  $(2 \times 2 \times 2 \text{ table})$ :

These represent conditional associations.

#### **Definition 2.4 (marginal table)**

The marginal table sums the partial tables:



This represents the **marginal association** (ignoring Z).

In general, let  $\mu_{ijk} =$  expected count in row i, col j, table k.

The conditional odds ratios,

$$\theta_{XY(k)} = \frac{\mu_{11k}\mu_{22k}}{\mu_{12k}\mu_{21k}}$$

which are estimated by

$$\hat{\theta}_{XY(k)} = \frac{n_{11k}n_{22k}}{n_{12k}n_{21k}}$$

The marginal odds ratio

$$\theta_{XY} = \frac{\mu_{11} + \mu_{22} + \mu_{12}}{\mu_{12} + \mu_{21} + \mu_{21}}$$

is estimated from the marginal table.

#### 2.2.2 Simpson's Paradox

Some counter-intuitive but possible situations:

- 1. There are conditional associations  $(\theta_{XY(k)} \neq 1)$  but no marginal association  $(\theta_{XY} = 1)$
- 2. There is a marginal association ( $\theta_{XY} \neq 1$ ) but no conditional associations ( $\theta_{XY(k)} = 1$ )
- 3. Simpson's paradox: The conditional associations are in the opposite direction from the marginal, e.g.

$$\theta_{XY(k)} > 1, \theta_{XY} < 1$$

	Full Population, ${f N}={f 52}$			Men (M), N = 20			Women ( $\neg$ M), $N = 32$		
	Success (S)	Failure (¬S)	Success Rate	Success	Failure	Success Rate	Success	Failure	Success Rate
Treatment (T)	20	20	50%	8	5	≈ 61%	12	15	≈ 44%
Control (¬T)	6	6	50%	4	3	≈ 57%	2	3	≈ 40%

Table 1: Simpson's Paradox: the type of association at the population level (positive, negative, independent) changes at the level of subpopulations. Numbers taken from Simpson's original example (1951).

Figure 2.1: Simpson's paradox

#### 2.2.3 Conditional Independence, Marginal Independence

#### Definition 2.5 (conditionally independent given Z, marginal independent)

We also call X and Y are conditionally independent given Z = k if  $\theta_{XY(k)} = 1$ . If this is true for all k, X and Y are conditionally independent given Z. Not the same to "X and Y are marginal independent if  $\theta_{XY} = 1$ ".

#### **Proposition 2.1**

For multinomial sampling, can show that conditional independence is

$$\pi_{ijk} = \frac{\pi_{i+k}\pi_{+jk}}{\pi_{++k}}, \quad \forall i, j, k$$

#### 2.2.4 Homogeneous Association

#### **Definition 2.6**

Let Z have K categories. X and Y have homogeneous association over Z if

$$\theta_{XY(1)} = \theta_{XY(2)} = \dots = \theta_{XY(K)}$$

(Conditional independence is a special case.)