

#### Statistics for Health Care

#### <u>Unit 4:</u>

Overview/Teasers

### Overview

 Probability distributions; expected value and variance; the binomial and normal distributions

### Teaser 1, Unit 4

- A 2012 Mega Millions lottery had a jackpot of \$656 million (\$474 immediate payout).
- Question I received: "If the odds of winning the Mega millions is 1 in 175,000,000 is there a significant statistical advantage in playing 100 picks rather than one?
- "For a half-billion dollars it almost seems worth it."

### Teaser 2, Unit 4

Imagine that you are in a resource-poor area and you want to screen the population for a fairly rare disease. But the antibody test is prohibitively expensive.

A clever cost-saving strategy is to pool the blood from multiple samples (using half of a person's blood sample and saving the other half). If the pooled lot is negative, this saves n-1 tests. If it's positive, then you go back and test each sample individually, requiring n+1 tests total.

If a particular disease has a prevalence of 10% in a population, will the pooling strategy save you tests? If so, what's the optimal number of samples to pool per lot?

### Teaser 3, Unit 4

- Ten patients with wrinkles were photographed before and after treatment with a new anti-aging treatment.
   An independent dermatologist was able to distinguish the pre and post photographs for 9 out of the 10 subjects.
- If the anti-aging treatment is completely ineffective, what's the probability that the dermatologist could have gotten at least 9 right purely by lucky guessing?



#### Statistics for Health Care

#### Module 1:

Probability distributions (functions)

### Probability function

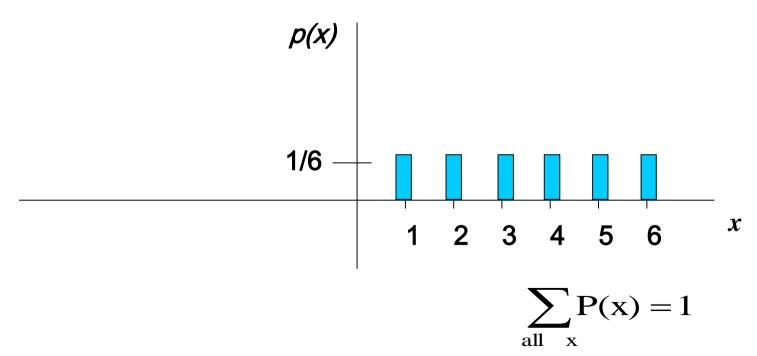
- Gives the probabilities of all possible outcomes.
- A mathematical function that maps each possible outcome x to its probability p(x).
- The probabilities must sum (or integrate) to 1.0.

# Probability functions can be discrete or continuous

- Discrete: can only take on certain values
  - <u>Examples</u>: Dead/alive, treatment/placebo, dice, whole numbers, counts, etc.
- **Continuous:** can theoretically take on any value within a given range (has an infinite continuum of possible values).
  - Examples: blood pressure, weight, the speed of a car, the real numbers from 1 to 6

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### Discrete example: roll of a die



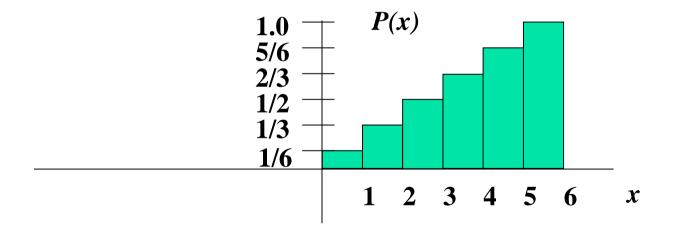


### Probability mass function (pmf)

X	p(x)
1	p(x=1)=1/6
2	<i>p(x=2)</i> =1/6
3	<i>p(x=3)</i> =1/6
4	<i>p(x=4)</i> =1/6
5	<i>p(x=5)</i> =1/6
6	<i>p(x=6)</i> =1/6

1.0

## Cumulative distribution function (CDF)



# Cumulative distribution function for a die

X	P(x≤A)
1	<i>P(x≤1)</i> =1/6
2	<i>P(x≤2)</i> =2/6
3	<i>P(x≤3)</i> =3/6
4	<i>P(x≤4)</i> =4/6
5	<i>P(x≤5)</i> =5/6
6	<i>P(x≤6)</i> =6/6

## Recall: blood types

#### Out of 100 donors . . . . .

84 donors are RH+	16 donors are RH-	
38 are O+	7 are O-	
34 are A+	6 are A-	
9 are B+	2 are B-	
3 are AB+	1 is AB-	

Source: AABB.ORG

# Probability distribution for blood types (discrete function):

<u>x</u>	<u>P(x)</u>
0	45%
A	40%
В	11%
AB	4%

#### **Practice Problem:**

The number of patients seen in the ER in any given hour is a random variable represented by x. The probability distribution for x is:

X	9	10	11	12	13
P(x)	.3	.3	.2	.1	.1

Find the probability that in a given hour:

- a. exactly 13 patients arrive p(x=13)=.1
- b. At least 12 patients arrive  $p(x \ge 12) = (.1 + .1) = .2$
- c. At most 11 patients arrive  $p(x \le 11) = (.3 + .3 + .2) = .80$

# Important discrete distributions in medical research:

- Binomial
  - Yes/no outcomes (dead/alive, treated/untreated, smoker/non-smoker, sick/well, etc.)
- Poisson
  - Counts (e.g., how many cases of disease in a given area)

#### Continuous case

- Any continuous mathematical function that integrates to 1 is a probability function.
- The probabilities associated with continuous functions are just areas under the curve (integrals!).
- Probabilities are given for a range of values, rather than a particular value.

### Continuous case

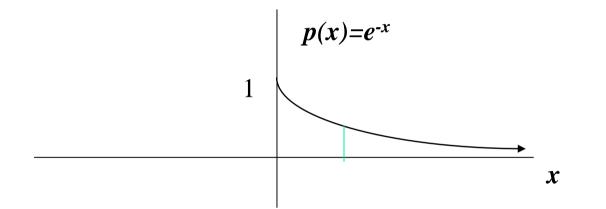
 For example, the exponential distribution is a continuous probability function, because the area under the curve is 1.0.

$$f(x) = e^{-x}$$

■ This function integrates to 1 :

$$\int_{0}^{+\infty} e^{-x} = -e^{-x} \quad \Big|_{0}^{+\infty} = 0 + 1 = 1$$

# Continuous case: "probability density function" (pdf)

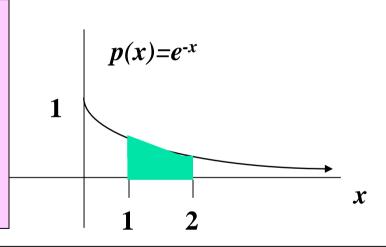


The probability that x is any exact particular value (such as 1.9976) is 0; we can only assign probabilities to possible ranges of x (=cumulative distribution function).

# Continuous case: Cumulative distribution function (CDF)

Clinical example: Imagine that survival times after lung transplant roughly follow an exponential function.

Then, the probability that a patient will die in the second year after surgery (between years 1 and 2) is 23%.



$$P(1 \le x \le 2) = \int_{1}^{2} e^{-x} = -e^{-x} \quad \Big|_{1}^{2} = -e^{-2} - -e^{-1} = -.135 + .368 = .23$$

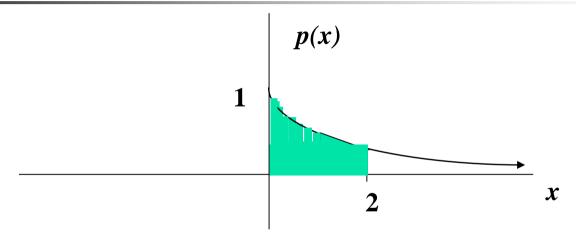
# Cumulative distribution function

As in the discrete case, we can specify the "cumulative distribution function" (CDF):

The CDF here =  $P(x \le A)$ =

$$\int_{0}^{A} e^{-x} = -e^{-x} \quad \Big|_{0}^{A} = -e^{-A} - -e^{0} = -e^{-A} + 1 = 1 - e^{-A}$$

### Example



$$P(x \le 2) = 1 - e^{-2} = 1 - .135 = .865$$

### Practice Problem

Suppose that survival drops off rapidly in the year following diagnosis of a certain type of advanced cancer. Suppose that the length of survival (or time-to-death) is a random variable that approximately follows an exponential distribution with parameter 2 (makes it a steeper drop off):

probability function : 
$$p(x = T) = 2e^{-2T}$$

$$note: \int_{0}^{+\infty} 2e^{-2x} = -e^{-2x} \Big|_{0}^{+\infty} = 0 + 1 = 1$$

What's the probability that a person who is diagnosed with this illness survives a year?

## Answer

The probability of dying within 1 year can be calculated using the cumulative distribution function:

Cumulative distribution function is:

$$P(x \le T) = -e^{-2x}$$
  $\Big|_{0}^{T} = 1 - e^{-2(T)}$ 

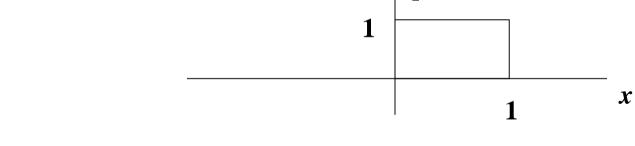
The chance of surviving past 1 year is:  $P(x \ge 1) = 1 - P(x \le 1)$ 

$$1 - (1 - e^{-2(1)}) = .135$$

# Example 2: Uniform distribution

The uniform distribution: all values are equally likely.

$$f(x)=1$$
, for  $1 \ge x \ge 0$ 

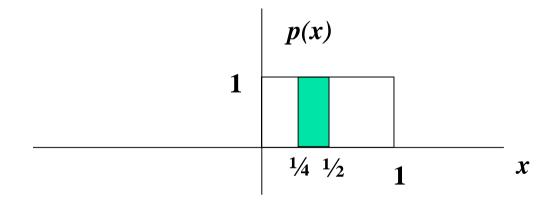


We can see it's a probability distribution because the area under the curve is 1:  $\int_{0}^{1} 1 = x \Big|_{0}^{1} = 1 - 0 = 1$ 

## 1

### Example: Uniform distribution

What's the probability that x is between  $\frac{1}{4}$  and  $\frac{1}{2}$ ?

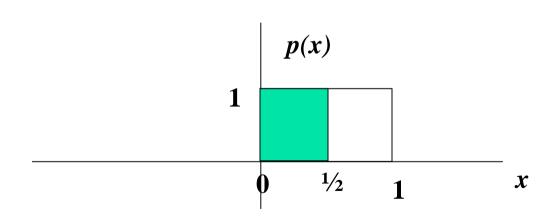


$$P(\frac{1}{2} \ge x \ge \frac{1}{4}) = \frac{1}{4}$$

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### Example: Uniform distribution

What's the probability that x is between 0 and  $\frac{1}{2}$ ?



$$P(\frac{1}{2} \ge x \ge 0) = \frac{1}{2}$$

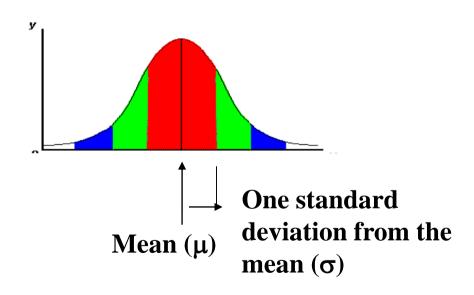
Clinical Research Example: When randomizing patients in an RCT, we often use a random number generator on the computer. These programs work by randomly generating a number between 0 and 1 (with equal probability of every number in between). Then a subject who gets X<.5 is control and a subject who gets X>.5 is treatment.



### Expected value and Variance

 All probability distributions are characterized by an expected value (mean) and a variance (standard deviation squared).







#### Statistics for Health Care

Module 2:

Expected value

### Expected value

- Expected value is just the mean (µ) of a probability distribution.
- It is a weighted average, calculated by weighting the value of each possible outcome by its probability.
- Expected value helps us make informed decisions based on how we expect x to behave on-average over the long-run.

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### Expected value, formally

Discrete case:

$$E(X) = \sum_{\text{all } x} x_i p(x_i)$$

**Continuous case:** 

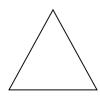
$$E(X) = \int_{\text{all } x} x_i p(x_i) dx$$

## 4

### Example: expected value

Recall the following probability distribution of ER arrivals:

X	9	10	11	12	13
P(x)	.3	.3	.2	.1	.1



$$\sum_{i=1}^{5} x_i p(x) = 9(.3) + 10(.3) + 11(.2) + 12(.1) + 13(.1) = 10.4$$

## A Sample Mean is a special case of Expected Value...

Sample mean, for a sample of n subjects: =

$$\overline{X} = \frac{\sum_{i=1}^{n} x_i}{n} = \sum_{i=1}^{n} x_i \left(\frac{1}{n}\right)$$

The probability (frequency) of each person in the sample is 1/n.

### Symbol Interlude

- $E(X) = \mu$ 
  - these symbols are used interchangeably



Expected value is an extremely useful concept for good decision-making!

# Example: the lottery

- The Lottery (also known as a tax on people who are bad at math...)
- A certain lottery works by picking 6 numbers from 1 to 49. It costs \$1.00 to play the lottery, and if you win, you win \$2 million after taxes.

If you play the lottery once, what are your expected winnings or losses?

# Lottery

+ 2 million

#### Calculate the probability of winning in 1 try:

1 _ 1	$=$ $\frac{1}{1}$ $=$ 7.2 x 10 <sup>-8</sup>
$\frac{(49)}{49!}$	$-\frac{13,983,816}{13,983,816}$
6 43!6!	

Out of 49 numbers, this is the number of distinct

combinations of 6.

"49 choose 6"

### The probability function (note, sums to 1.0):

<b>x\$</b>	p(x)
-1	.99999928
. 0 '111'	7.0 40.8

## **Expected Value**

#### The probability function

x\$	p(x)
-1	.99999928
+ 2 million	7.2 x 10 <sup>-8</sup>

#### **Expected Value**

$$E(X) = P(win)*\$2,000,000 + P(lose)*-\$1.00$$

$$= 2.0 \times 10^6 * 7.2 \times 10^{-8} + .9999999928 (-1) = .144 - .9999999998 = -\$.86$$

Negative expected value is never good!

You shouldn't play if you expect to lose money!

# Expected Value

If you play the lottery every week for 10 years, what are your expected winnings or losses?

$$520 \times (-.86) = -\$447.20$$

# 2012 record Mega Millions jackpot...

- 2012 Mega Millions had a jackpot of \$656 million (\$474 immediate payout).
- Question I received: "If the odds of winning the Mega millions is 1 in 175,000,000 is there a significant statistical advantage in playing 100 quick picks rather than one?
- "For a half-billion-with-a-B dollars it almost seems worth it."

### Expected value for 1 ticket:

Chances of losing, 1 ticket: 1-1/175,000,000=99.9999994%

x\$	p(x)
-1	. 99999994
+ 500 million	6 × 10 <sup>-9</sup>

#### **Expected Value**

$$E(X) = P(win)*\$500,000,000 + P(lose)*-\$1.00$$
  
= 6.0 x 10<sup>-9</sup> \* 500,000,000+ .999999994 (-1) = +2

# Answer, 100 tickets:

Chances of losing, 100 tickets: 99.99943%

<b>x\$</b>	p(x)			
-100	. 9999943			
+ 500 million	5.7× 10 <sup>-7</sup>			

#### **Expected Value**

$$E(X) = P(win)*\$500,000,000 + P(lose)*-\$100$$
  
= 5.7 x 10<sup>-7</sup> \* 500,000,000+ .99999943 (-100) = +185

# \_\_\_\_So...

- One could make a case for playing!
- You can work out that the expected payout only has to be >\$176 million for expected value to be positive (for either 1 ticket or 100 tickets).
- BUT...

# BUT then consider the high chance of multiple winners!

- When the jackpot is huge, lots of people play. The chance of multiple winners (who will share the jackpot) is quite high!
- Assume 600 million tickets are sold, then the probability distribution here is (where x is the number of winners):

X	0	1	2	3	4	5	6	7	8	9	10
P(x)	.03	.11	.19	.22	.19	.13	.07	.036	.015	.005	.002

- E(X)=0+1\*.11+2\*.19+3\*.22+4\*.19+5\*.13+6\*.07+7\*.036+8\*.015+ 9\*.005+10\*.002=3.4
- Therefore, the expected winnings if you win are actually 500 million/3.4=147 million

### Not to mention taxes!

- You can also assume that about half is going to be lost in taxes.
- And, the fact is, you're still going to lose with almost near certainty!
  - Probability 99.9999...%!

# Gambling (or how casinos can afford to give so many free drinks...)

A roulette wheel has the numbers 1 through 36, as well as 0 and 00. If you bet \$1 that an odd number comes up, you win or lose \$1 according to whether or not that event occurs. If random variable X denotes your net gain, X=1 with probability 18/38 and X=-1 with probability 20/38.

$$E(X) = 1(18/38) - 1(20/38) = -\$.053$$

∴On average, the casino wins (and the player loses) 5 cents per game.

The casino rakes in even more if the stakes are higher:

$$E(X) = 10(18/38) - 10(20/38) = -\$.53$$

If the cost is \$10 per game, the casino wins an average of 53 cents per game. If 10,000 games are played in a night, that's a cool \$5300.

## Challenge Problem

- Imagine that you are in a resource-poor area and you want to screen the population for a fairly rare disease. But the antibody test is prohibitively expensive.
- A clever cost-saving strategy is to pool the blood from multiple samples (using half of a person's blood sample and saving the other half). If the pooled lot is negative, this saves *n-1* tests. If it's positive, then you go back and test each sample individually, requiring *n+1* tests total.
- If a particular disease has a prevalence of 10% in a population, will the pooling strategy save you tests? If so, what's the optimal number of samples to pool per lot?
- Solve by "brute force" assuming you want to screen 100 people.

# Try pooling 20...

If you pool 20 samples at a time (5 lots), how many tests do you expect to have to run (assuming the test is perfect!)?

## Pooling 20...

If you pool 20 samples at a time (5 lots), how many tests do you expect to have to run (assuming the test is perfect!)?

X = the number of tests you have to run per lot:

E(X) = P(pooled lot is negative)(1) + P(pooled lot is positive) (21)

$$E(X) = (.90)^{20} (1) + [1-.90^{20}] (21) = 12.2\% (1) + 87.8\% (21) = 18.56$$

E(total number of tests) = 5\*18.56 = 92.8

# Pooling 10...

What if you pool only 10 samples at a time?

$$E(X) = (.90)^{10} (1) + [1-.90^{10}] (11) = 35\% (1) + 65\% (11) = 7.5$$
 average per lot

10 lots \* 7.5 = 75

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### Pooling 5...

5 samples at a time?

$$E(X) = (.90)^5 (1) + [1-.90^5] (6) = 59\% (1) + 41\% (6) = 3.05$$
 average per lot

20 lots \* 3.05 = 61

# Pooli

### Pooling 4...

4 samples at a time?

$$E(X) = (.90)^4 (1) + [1-.90^4] (5) = 2.38$$
 average per lot

$$25 lots * 2.38 = 59$$

# Pooling 3...

3 samples at a time?

$$E(X) = (.90)^3 (1) + [1-.90^3] (4) = 1.81$$
 average per lot

$$33 lots * 1.81 = 60$$

### Extension to continuous case:

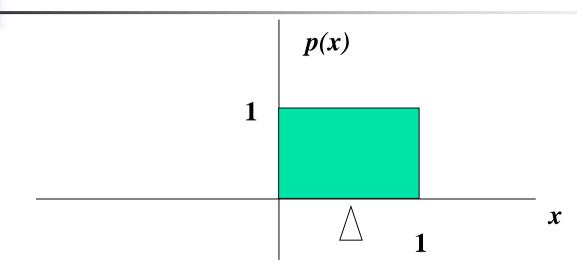
Discrete case:

$$E(X) = \sum_{\text{all } x} x_i p(x_i)$$

**Continuous case:** 

$$E(X) = \int_{\text{all } x} x_i p(x_i) dx$$

# Extension to continuous case: uniform distribution



$$E(X) = \int_{0}^{1} x(1) dx = \frac{x^{2}}{2} \Big|_{0}^{1} = \frac{1}{2} - 0 = \frac{1}{2}$$



### Statistics for Health Care

Module 3:

Variance



The variance (or standard deviation) quantifies the variability of a probability distribution.

Variance/standard deviation is calculated similarly to how we calculated variance/standard deviation for sample data. However, outcomes are weighted by their probabilities.

## Variance

Variance=the average squared distance from the mean

$$\sigma^{2} = Var(x) = \sum_{\text{all } x} (x_{i} - \mu)^{2} p(x_{i})$$

## Example: variance

Find the variance and standard deviation for the number of patients to arrive in the ER (recall that the mean is 10.4).

mean is 10.4).	X	9	10	11	12	13	
	P(x)	.3	.3	.2	.1	.1	

$$Var(x) = \sum_{i=1}^{3} (x_i - 10.4)^2 * p(x_i) = (1.4^2)(.3) + (0.4^2)(.3) + (0.6^2)(.2) + (1.6^2)(.1) + (2.6^2)(.1) = 1.64$$

$$SD(x) = \sqrt{1.64} = 1.28$$

Interpretation: In an average hour, we expect 10.4 patients to arrive in the ER, plus or minus 1.28. This gives you a feel for what would be considered a typical hour!

## Variance, formally

#### Discrete case:

$$Var(X) = \sum_{\text{all x}} (x_i - \mu)^2 p(x_i)$$

#### **Continuous case:**

$$Var(X) = \int_{-\infty}^{\infty} (x_i - \mu)^2 p(x_i) dx$$

# Similarity to empirical variance

The variance of a sample:  $S^2 =$ 

$$\frac{\sum_{i=1}^{N} (x_i - \bar{x})^2}{n-1} = \sum_{i=1}^{N} (x_i - \bar{x})^2 (\frac{1}{n-1})$$

Division by n-1 reflects the fact that we have lost a "degree of freedom" (piece of information) because we had to estimate the sample mean before we could estimate the sample variance.

### **Practice Problem**

A roulette wheel has the numbers 1 through 36, as well as 0 and 00. If you bet \$1.00 that an odd number comes up, you win or lose \$1.00 according to whether or not that event occurs. If X denotes your net gain, X=1 with probability 18/38 and X=-1 with probability 20/38.

We already calculated the mean to be = -\$.053.

What are the variance and standard deviation of X?

### Answer

$$\sigma^{2} = \sum_{\text{all } x} (x_{i} - \mu)^{2} p(x_{i})$$

$$= (+1 - -0.053)^{2} (18/38) + (-1 - 0.053)^{2} (20/38)$$

$$= (1.053)^{2} (18/38) + (-1 + 0.053)^{2} (20/38)$$

$$= (1.053)^{2} (18/38) + (-0.947)^{2} (20/38)$$

$$= .997$$

$$\sigma = \sqrt{.997} = .99$$

Standard deviation is \$.99. Interpretation: On average, you're either 1 dollar above or 1 dollar below the mean, which is just under zero. Makes sense!

# -

# \*\*A few notes about Variance as a mathematical operator:

- If c= a constant number (i.e., not a variable) and X and Y are random variables, then Var(c) = 0
- Var (c+X)= Var(X)
- $Var(cX) = c^2 Var(X)$
- Var(X+Y)= Var(X) + Var(Y) ONLY IF X and Y are independent!!!!

# Var(c) = 0

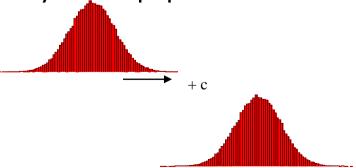
Var(c) = 0

Constants don't vary!

# Var (c+X) = Var(X)

$$Var (c+X) = Var(X)$$

Adding a constant to every instance of a random variable doesn't change the variability. It just shifts the whole distribution by c. If everybody grew 5 inches suddenly, the variability in the population would still be the same.



# $Var(cX) = c^2 Var(X)$

 $Var(cX) = c^2Var(X)$ 

Multiplying each instance of the random variable by c makes it c-times as wide of a distribution, which corresponds to c<sup>2</sup> as much variance (deviation squared). For example, if everyone suddenly became twice as tall, there'd be twice the deviation and 4 times the variance in heights in the population.

# Var(X+Y)=Var(X)+Var(Y)

Var(X+Y)= Var(X) + Var(Y) ONLY IF X and Y are independent!!!!!!!



### Statistics for Health Care

### Module 4:

The binomial distribution

# Binomial Probability Distribution

- A fixed number of trials, n
  - e.g., 15 tosses of a coin; 20 patients; 1000 people surveyed
- A binary outcome
  - e.g., head or tail in each toss of a coin; disease or no disease
  - Probability of "success" is p, probability of "failure" is 1 p
- Constant probability for each trial
  - e.g., Probability of getting a tail is the same each time we toss the coin



Take the example of 5 coin tosses. What's the probability that you flip exactly 3 heads in 5 coin tosses?

### Binomial distribution

Solution:

One way to get exactly 3 heads: HHHTT

What's the probability of this <u>exact</u> arrangement?  $P(heads)xP(heads)xP(heads)xP(heads)xP(tails)xP(tails) = (1/2)^3 x (1/2)^2$ 

Another way to get exactly 3 heads: THHHT Probability of this exact outcome =  $(1/2)^1 x (1/2)^3 x (1/2)^1 = (1/2)^3 x (1/2)^2$ 

### Binomial distribution

In fact,  $(1/2)^3 x (1/2)^2$  is the probability of each unique outcome that has exactly 3 heads and 2 tails.

So, the overall probability of 3 heads and 2 tails is:  $(1/2)^3 x (1/2)^2 + (1/2)^3 x (1/2)^2 + (1/2)^3 x (1/2)^2 + ....$  for as many unique arrangements as there are—but how many are there??

Outcome THHHT HHHTT TTHHH HTTHH HHTTH HTHHT THTHH HTHHT THTHH HTHTH	Probability $(1/2)^{3} x (1/2)^{2}$
HHTHT <u>THHTH</u>	$ \frac{(1/2)^3 x (1/2)^2}{(1/2)^3 x (1/2)^2} $
10 arrangements $x$ (1	$(1/2)^3 x (1/2)^2$
	THHHT HHHTT TTHHH HTTHH HHTTH HTHHT THTHH HTHHT HHHTH

The probability of each unique outcome (note: they are all equal)

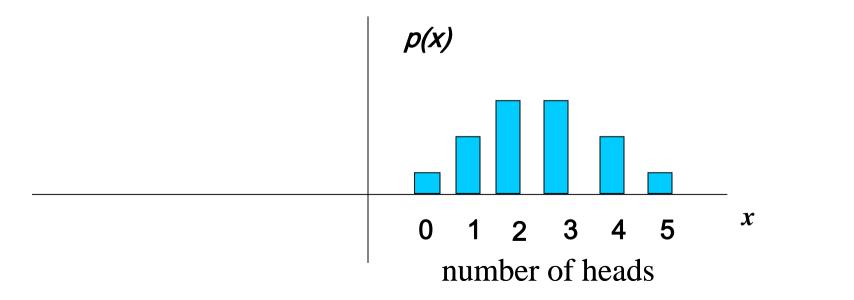
∴ P(3 heads and 2 tails) = 
$$\binom{5}{3}$$
  $x$  P(heads)<sup>3</sup>  $x$  P(tails)<sup>2</sup> =

$$10 \times (1/2)^{5=3}1.25\%$$

# 4

### Binomial distribution function

X= the number of heads tossed in 5 coin tosses



## Example 2

As voters exit the polls, you ask a representative random sample of 6 voters if they voted for a candidate A. If the true percentage of voters who vote for the candidate A is 55.1%, what is the probability that, *in your sample*, exactly 2 voted for the candidate A and 4 did not?

# Solution:

voters

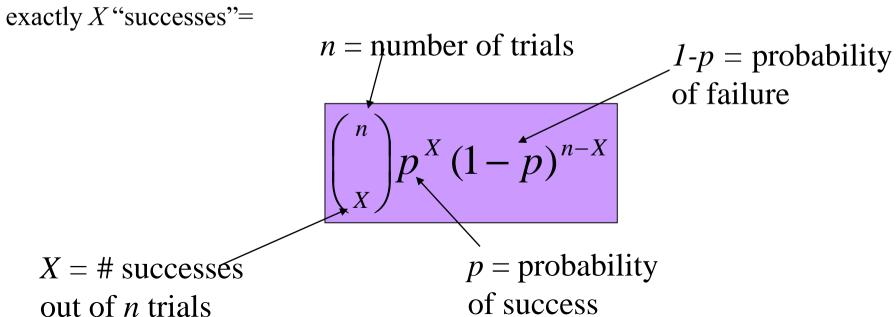
```
Probability
              Outcome
              YYNNNN
                                                              = (.551)^2 x (.449)^4
                                (.449)^1 x (.551)^2 x (.449)^3 = (.551)^2 x (.449)^4
              NYYNNN
                                (.449)^2 x (.551)^2 x (.449)^2 = (.551)^2 x (.449)^4
              NNYYNN
ways to
                                (.449)^3 x (.551)^2 x (.449)^1 = (.551)^2 x (.449)^4
              NNNYYN
arrange 2 Prop
                                (.449)^4 x (.551)^2
                                                              = (.551)^2 x (.449)^4
100 votes
              NNNNYY
among 6
```

15 arrangements  $x (.551)^2 x (.449)^4$ 

:.P(2 yes votes exactly) = 
$$\binom{6}{2}x$$
 (.551)<sup>2</sup> x (.449)<sup>4</sup> = 18.5%

## Binomial distribution, generally

Note the general pattern emerging  $\rightarrow$  if you have only two possible outcomes (call them 1/0 or yes/no or success/failure) in *n* independent trials, then the probability of exactly *X* "successes"=



### **Binomial**

- We write: X ~ Bin (n, p)
  - Read as: "X is distributed binomially with parameters n and p
- And the probability that there are <u>exactly</u> X successes is:

$$P(X) = \binom{n}{X} p^{X} (1-p)^{n-X}$$



## Binomial distribution: example

- Ten patients with wrinkles were photographed before and after treatment with a new anti-aging treatment. An independent dermatologist was able to distinguish the pre and post photographs for 9 out of the 10 subjects.
- If the anti-aging treatment is completely ineffective, what's the probability that the dermatologist could have gotten at least 9 right purely by lucky guessing?

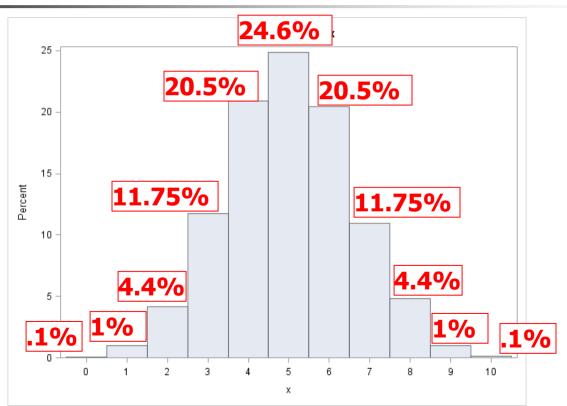
# Example

#### $X \sim Bin (10, 0.5)$ $P(X \ge 9) = P(X = 9) + P(X = 10)$

$$P(X \ge 9) = \binom{10}{9} (.5)^9 (1 - .5)^1 + \binom{10}{10} (.5)^{10} (1 - .5)^0$$

$$= \frac{10!}{9!1!} (.5)^9 (.5)^1 + \frac{10!}{10!0!} (.5)^{10} = 10 * (.5)^9 + (.5)^{10} = 0.01 + .001 = 0.011$$

### The full probability distribution:

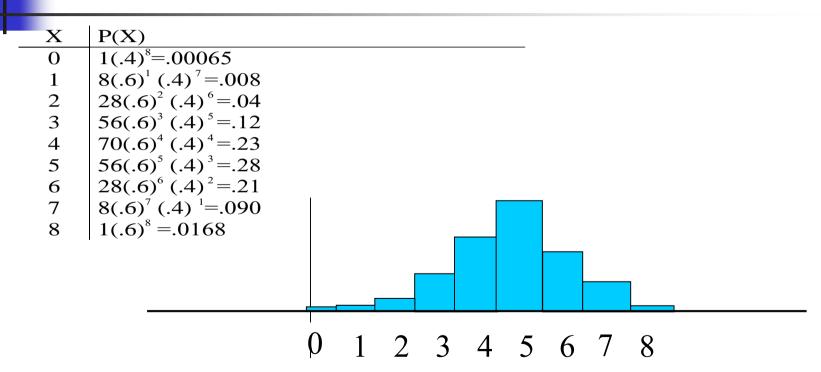




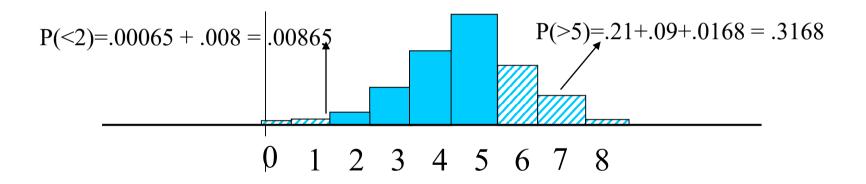
You are conducting a case-control study of smoking and lung cancer. If the probability of being a smoker among lung cancer cases is .6, what's the probability that in a group of 8 cases you have:

- a. Less than 2 smokers?
- b. More than 5?

#### Answer



# Answer, continued



# \*\*All probability distributions are characterized by an expected value and a variance:

If X follows a binomial distribution with parameters n and p:  $X \sim Bin(n, p)$ 

#### Then:

$$E(X) = np$$

$$Var(X) = np(1-p)$$

$$SD(X) = \sqrt{np(1-p)}$$

Note: the variance will always lie between

0\*N ~ 0.25 \*N

p(1-p) reaches maximum at p=.5

P(1-p)=.25



You flip a coin 100 times. What are the expected value, variance, and standard deviation for the number of heads?

#### Answer

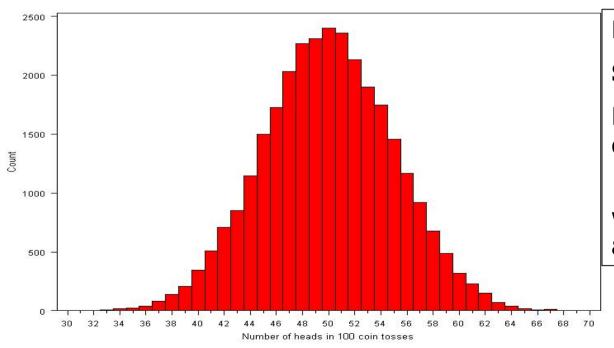
$$E(X) = 100 (.5) = 50$$
  
 $Var(X) = 100 (.5) (.5) = 25$   
 $SD(X) = square root (25) = 5$ 

Interpretation: When we toss a coin 100 times, we expect to get 50 heads plus or minus 5.

## Or use computer simulation...

- Flip coins virtually!
  - Flip a virtual coin 100 times; count the number of heads.
  - Repeat this over and over again a large number of times (we'll try 30,000 repeats!)
  - Plot the 30,000 results.

### Coin tosses...



Mean = 50

Std. dev = 5

Follows a normal distribution

∴95% of the time, we get between 40 and 60 heads...



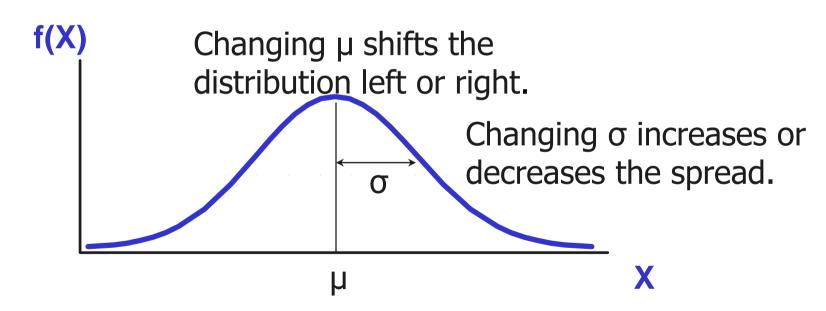
#### Statistics for Health Care

#### Module 5:

The normal and standard normal distributions



#### The Normal Distribution



# The Normal Distribution: as mathematical function (pdf)

$$f(x) = \frac{1}{\sigma \sqrt{2\pi}} \cdot e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2}$$

Note constants:

 $\pi$ =3.14159 e=2.71828

curve with different centers and spreads depending on  $\mu$  and  $\sigma$ 

This is a bell shaped

### The Normal PDF

It's a probability function, so no matter what the values of  $\mu$  and  $\sigma$ , must integrate to 1!

$$\int_{-\infty}^{+\infty} \frac{1}{\sigma \sqrt{2\pi}} \cdot e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2} dx = 1$$

# Normal distribution is defined by its mean and standard dev.

$$\mathsf{E}(\mathsf{X}) = \mu = \int_{-\infty}^{+\infty} x \frac{1}{\sigma \sqrt{2\pi}} \cdot e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2} dx$$

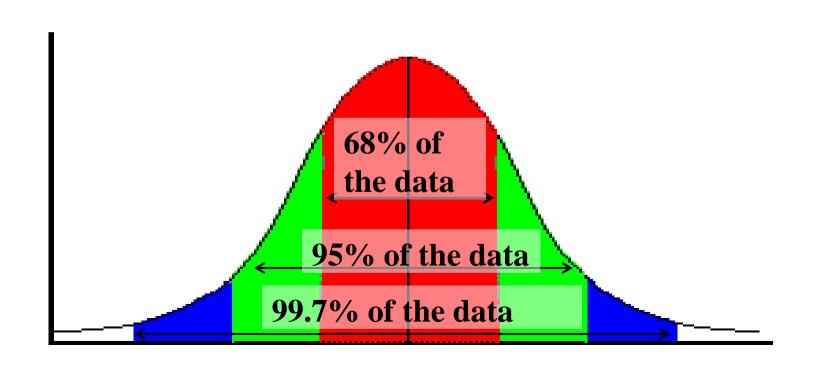
$$Var(X) = \sigma^2 = \int_{-\infty}^{+\infty} (x - \mu)^2 \cdot \frac{1}{\sigma \sqrt{2\pi}} \cdot e^{\frac{-1}{2}(\frac{x - \mu}{\sigma})^2} dx$$

Standard Deviation(X)= $\sigma$ 

# Recall: 68-95-99.7 Rule

No matter what  $\mu$  and  $\sigma$  are, the area between  $\mu$ - $\sigma$  and  $\mu$ + $\sigma$  is about 68%; the area between  $\mu$ -2 $\sigma$  and  $\mu$ +2 $\sigma$  is about 95%; and the area between  $\mu$ -3 $\sigma$  and  $\mu$ +3 $\sigma$  is about 99.7%. Almost all values fall within 3 standard deviations.





# •

### 68-95-99.7 Rule in Math!

$$\int_{\mu-\sigma}^{\mu+\sigma} \frac{1}{\sigma\sqrt{2\pi}} \bullet e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2} dx = .68$$

$$\int_{\mu-2\sigma}^{\mu+2\sigma} \frac{1}{\sigma\sqrt{2\pi}} \bullet e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2} dx = .95$$

$$\int_{\mu-3\sigma}^{\mu+3\sigma} \frac{1}{\sigma\sqrt{2\pi}} \bullet e^{-\frac{1}{2}(\frac{x-\mu}{\sigma})^2} dx = .997$$



- Suppose SAT scores roughly follows a normal distribution in the U.S. population of college-bound students (with range restricted to 200-800), and the average math SAT is 500 with a standard deviation of 50, then:
  - 68% of students will have scores between 450 and 550
  - 95% will be between 400 and 600
  - 99.7% will be between 350 and 650



# ■ BUT: What's the probability of getting a math SAT score of 575 or less, $\mu$ =500 and $\sigma$ =50?

#### 68-95-99.7 rule doesn't help here!

$$\int_{-\infty}^{575} \frac{1}{50\sqrt{2\pi}} \cdot e^{-\frac{1}{2}(\frac{x-500}{50})^2} dx = ?$$
 Solve this integral? No thanks!



# The Standard Normal (Z): "Universal Currency"

The standard normal curve has a mean of 0 and a standard deviation of 1.

$$p(Z) = \frac{1}{(1)\sqrt{2\pi}} \cdot e^{-\frac{1}{2}(\frac{Z-0}{1})^2} = \frac{1}{\sqrt{2\pi}} \cdot e^{-\frac{1}{2}(Z)^2}$$

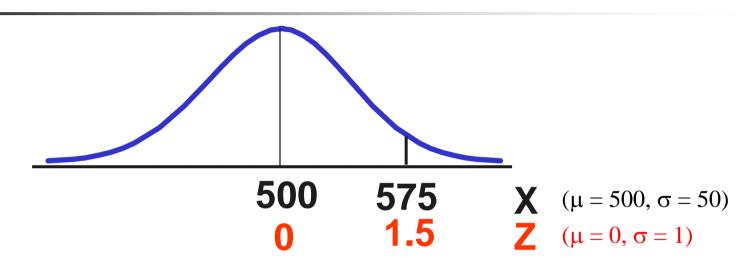
## The Standard Normal Distribution (Z)

All normal distributions can be converted into the standard deviation units ("Z-scores") by subtracting the mean and dividing by the standard deviation:

$$Z = \frac{X - \mu}{\sigma}$$

Standard deviation units! Universal currency!

# Converting to the standard normal...



$$Z = \frac{575 - 500}{50} = 1.5$$

## Example

• What's the probability of getting a math SAT score of 575 or less,  $\mu$ =500 and  $\sigma$ =50?

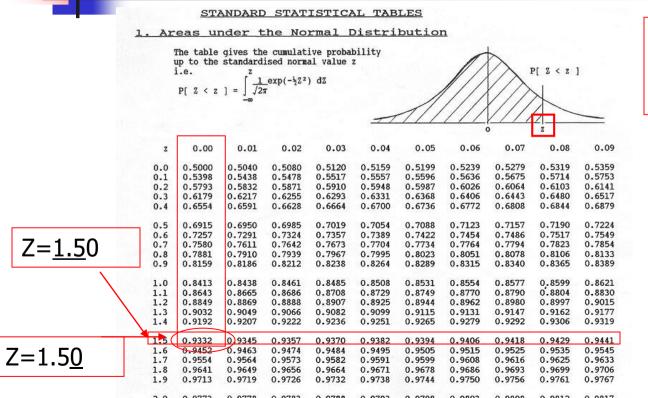
$$Z = \frac{575 - 500}{50} = 1.5$$

•i.e., A score of 575 is 1.5 standard deviations above the mean

# Standard Normal Charts

- Someone integrated all the areas under the standard normal curve and put them in a chart.
- Look up Z= 1.5 in standard normal chart → .9332

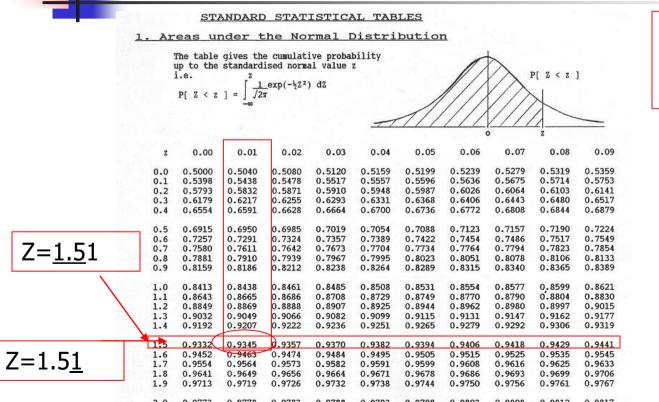
# Looking up probabilities in the standard normal table



What is the area to the left of Z=1.50 in a standard normal curve?

Area is 93.32%

# Looking up probabilities in the standard normal table



What is the area to the left of Z=1.51 in a standard normal curve?

Area is 93.45%

# Practice problem

- If birth weights in a population are normally distributed with a mean of 109 oz and a standard deviation of 13 oz,
- What is the chance of obtaining a birth weight of 141 oz or heavier when sampling birth records at random?
- What is the chance of obtaining a birth weight of 120 or lighter?

## Answer

What is the chance of obtaining a birth weight of 141 oz *or heavier* when sampling birth records at random?

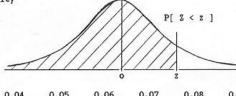
$$Z = \frac{141 - 109}{13} = 2.46$$

#### STANDARD STATISTICAL TABLES

#### 1. Areas under the Normal Distribution

The table gives the cumulative probability up to the standardised normal value z i.e. z

1.e. 
$$z = \int_{-\infty}^{z} \frac{1}{\sqrt{2\pi}} \exp(-\frac{1}{2}Z^{2}) dZ$$



							Ó		Z	
z	0.00	0.01	0.02	0.03	0.04	0.05	0.06	0.07	0.08	0.0
0.0	0.5000	0.5040	0.5080	0.5120	0.5159	0.5199	0.5239	0.5279	0.5319	0.535
0.1	0.5398	0.5438	0.5478	0.5517	0.5557	0.5596	0.5636	0.5675	0.5714	0.575
0.2	0.5793	0.5832	0.5871	0.5910	0.5948	0.5987	0.6026	0.6064	0.6103	0.614
0.3	0.6179	0.6217	0.6255	0.6293	0.6331	0.6368	0.6406	0.6443	0.6480	0.651
0.4	0.6554	0.6591	0.6628	0.6664	0.6700	0.6736	0.6772	0.6808	0.6844	0.687
0.5	0.6915	0.6950	0.6985	0.7019	0.7054	0.7088	0.7123	0.7157	0.7190	0.722
0.6	0.7257	0.7291	0.7324	0.7357	0.7389	0.7422	0.7454	0.7486	0.7517	0.754
0.7	0.7580	0.7611	0.7642	0.7673	0.7704	0.7734	0.7764	0.7794	0.7823	0.785
0.8	0.7881	0.7910	0.7939	0.7967	0.7995	0.8023	0.8051	0.8078	0.8106	0.813
0.9	0.8159	0.8186	0.8212	0.8238	0.8264	0.8289	0.8315	0.8340	0.8365	0.8389
1.0	0.8413	0.8438	0.8461	0.8485	0.8508	0.8531	0.8554	0.8577	0.8599	0.862
1.1	0.8643	0.8665	0.8686	0.8708	0.8729	0.8749	0.8770	0.8790	0.8804	0.883
1.2	0.8849	0.8869	0.8888	0.8907	0.8925	0.8944	0.8962	0.8980	0.8997	0.901
1.3	0.9032	0.9049	0.9066	0.9082	0.9099	0.9115	0.9131	0.9147	0.9162	0.917
1.4	0.9192	0.9207	0.9222	0.9236	0.9251	0.9265	0.9279	0.9292	0.9306	0.9319
1.5	0.9332	0.9345	0.9357	0.9370	0.9382	0.9394	0.9406	0.9418	0.9429	0.944
1.6	0.9452	0.9463	0.9474	0.9484	0.9495	0.9505	0.9515	0.9525	0.9535	0.954
1.7	0.9554	0.9564	0.9573	0.9582	0.9591	0.9599	0.9608	0.9616	0.9625	0.9633
1.8	0.9641	0.9649	0.9656	0.9664	0.9671	0.9678	0.9686	0.9693	0.9699	0.970
1.9	0.9713	0.9719	0.9726	0.9732	0.9738	0.9744	0.9750	0.9756	0.9761	0.976
2.0	0.9773	0.9778	0.9783	0.9788	0.9793	0.9798	0.9803	0.9808	0.9812	0.981
2.1	0.9821	0.9826	0.9830	0.9834	0.9838	0.9842	0.9846	0.9850	0.9854	0.985
2.2	0.9861	0.9865	0.9868	0.9871	0.9874	0.9878	0.9881	0.9884	0.9887	0.9890
2.3	0.9893	0.9896	0.9898	0.9901	0.9904	0.9906	0.9909	0.9911	0.9913	0.991
2.4	0.9918	0.9920	0.9922	0.9924	0.9927	0.9929	0.9931	0.9932	0.9934	0.993
2.5	0.9938	0.9940	0.9941	0.9943	0.9945	0.9946	0.9948	0.9949	0.9951	0.995
2.6	0.9953	0.9955	0.9956	0.9957	0.9959	0.9960	0.9961	0.9962	0.9963	0.996
2.7	0.9965	0.9966	0.9967	0.9968	0.9969	0.9970	0.9971	0.9972	0.9973	0.997
2.8	0.9974	0.9975	0.9976	0.9977	0.9977	0.9978	0.9979	0.9980	0.9980	0.998
2.9	0.9981	0.9982	0.9982	0.9983	0.9984	0.9984	0.9985	0.9985	0.9986	0.998
z	3.00	3.10	3.20	3.30	3.40	3.50	3.60	3.70	3.80	3.90
P	0.9986	0.9990	0.9993	0.9995	0.9997	0.9998	0.9998	0.9999	0.9999	1.0000

Area to the left of Z=2.46 is .9931

Area to the right of 2.46 is: 1-.9931 = .0069 or .69%

# Answer

b. What is the chance of obtaining a birth weight of 120 *or lighter*?

$$Z = \frac{120 - 109}{13} = .85$$

### Areas under the Normal Distribution

The table gives the cumulative probability

0.9713

0.9773

2.0

0.9719

0.9778

0.9726

0.9783

0.9732

0.9788

0.9738

0.9793

0.9744

0.9798

0.9750

0.9803

0.9756

0.9808

0.9761

0.9812

0.9767

0.9817

up to the standardised normal value z P[ Z < z ] i.e. 1 exp(-322) dZ P[Z < z] =0.01 0.02 0.03 0.04 0.05 0.06 0.07 0.08 0.09 0.00 Z 0.5120 0.5159 0.5199 0.5239 0.5279 0.5319 0.5359 0.0 0.5000 0.5040 0.5080 0.1 0.5398 0.5438 0.5478 0.5517 0.5557 0.5596 0.5636 0.5675 0.5714 0.5753 0.2 0.5793 0.5832 0.5871 0.5910 0.5948 0.5987 0.6026 0.6064 0.6103 0.6141 0.6293 0.6406 0.6443 0.6480 0.6517 0.3 0.6179 0.6217 0.6255 0.6331 0.6368 0.6554 0.6591 0.6628 0.6664 0.6700 0.6736 0.6772 0.6808 0.6844 0.6879 0.4 0.5 0.6915 0.6950 0.6985 0.7019 0.7054 0.7088 0.7123 0.7157 0.7190 0.7224 0.6 0.7257 0.7291 0.7324 0.7357 0.7389 0.7422 0.7454 0.7486 0.7517 0.7549 0.7580 0.7611 0.7642 0.7673 0.7704 0.7734 0.7764 0.7794 0.7823 0.7854 0.7 0.7881 0.7910 0.7939 0.7967 0.7995 0.8023 0.8051 0.8078 0.8106 0.8133 0.8 0.9 0.8159 0.8186 0.8212 0.8238 0.8264 0.8289 0.8315 0.8340 0.8365 0.8389 1.0 0.8413 0.8438 0.8461 0.8485 0.8508 0.8531 0.8554 0.8577 0.8599 0.8621 1.1 0.8643 0.8665 0.8686 0.8708 0.8729 0.8749 0.8770 0.8790 0.8804 0.8830 0.8849 0.8869 0.8888 0.8907 0.8925 0.8944 0.8962 0.8980 0.8997 0.9015 0.9177 0.9032 0.9049 0.9066 0.9082 0.9099 0.9115 0.9131 0.9147 0.9162 0.9192 0.9207 0.9222 0.9236 0.9251 0.9265 0.9279 0.9292 0.9306 0.9319 1.5 0.9332 0.9345 0.9357 0.9370 0.9382 0.9394 0.9406 0.9418 0.9429 0.9441 1.6 0.9452 0.9463 0.9474 0.9484 0.9495 0.9505 0.9515 0.9525 0.9535 0.9545 0.9554 0.9564 0.9573 0.9582 0.9591 0.9599 0.9608 0.9616 0.9625 0.9633 0.9641 0.9649 0.9656 0.9664 0.9671 0.9678 0.9686 0.9693 0.9699 0.9706

Area to the left of Z=0.85 is .8023 or 80.23%.



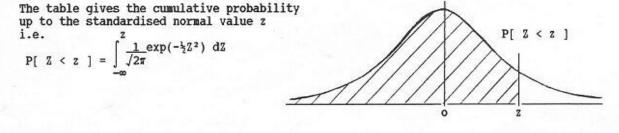
 $\phi$ (area)= Z: gives the Z-value that goes with the probability you want

For example, what if you wanted to know the math SAT score that corresponding to the 90<sup>th</sup> percentile (assuming a mean of 50 and a standard deviation of 50)?

In the Table, find the Z-value that corresponds to an area of 90%...

#### STANDARD STATISTICAL TABLES

#### Areas under the Normal Distribution



	P[ Z < z	$] = \int \sqrt{\frac{1}{2}}$	Zexb( 52	, 42		- //	////		X		
						1//	////	///			
					7	///	///	<u>/ / / /</u>	Z		
z	0.00	0.01	0.02	0.03	0.04	0.05	0.06	0.07	0.08	0.09	
0.0	0.5000	0.5040	0.5080	0.5120	0.5159	0.5199	0.5239	0.5279	0.5319	0.5359	
0.1	0.5398	0.5438	0.5478	0.5517	0.5557	0.5596	0.5636	0.5675	0.5714	0.5753	
0.2	0.5793	0.5832	0.5871	0.5910	0.5948	0.5987	0.6026	0.6064	0.6103	0.6141	
0.3	0.6179	0.6217	0.6255	0.6293	0.6331	0.6368	0.6406	0.6443	0.6480	0.6517	
0.4	0.6554	0.6591	0.6628	0.6664	0.6700	0.6736	0.6772	0.6808	0.6844	0.6879	
0.5	0.6915	0.6950	0.6985	0.7019	0.7054	0.7088	0.7123	0.7157	0.7190	0.7224	
0.6	0.7257	0.7291	0.7324	0.7357	0.7389	0.7422	0.7454	0.7486	0.7517	0.7549	
0.7	0.7580	0.7611	0.7642	0.7673	0.7704	0.7734	0.7764	0.7794	0.7823	0.7854	
0.8	0.7881	0.7910	0.7939	0.7967	0.7995	0.8023	0.8051	0.8078	0.8106	0.8133	
0.9	0.8159	0.8186	0.8212	0.8238	0.8264	0.8289	0.8315	0.8340	0.8365	0.8389	
1.0	0.8413	0.8438	0.8461	0.8485	0.8508	0.8531	0.8554	0.8577	0.8599	0.8621	
1.1	0.8643	0.8665	0.8686	0.8708	0.8729	0.8749	0.8770	0.8790	0.8804	0.8830	
1.2	0.8849	0.8869	0.8888	0.8907	0.8925	0.8944	0.8962	0.8980	0.8997	0.9015	
1.3	0.9032	0.9049	0.9066	0.9082	0.9099	0.9115	0.9131	0.9147	0.9162	0.9177	
1.4	0.9192	0.9207	0.9222	0.9236	0.9251	0.9265	0.9279	0.9292	0.9306	0.9319	
1.5	0.9332	0.9345	0.9357	0.9370	0.9382	0.9394	0.9406	0.9418	0.9429	0.9441	
1.6	0.9452	0.9463	0.9474	0.9484	0.9495	0.9505	0.9515	0.9525	0.9535	0.9545	
1.7	0.9554	0.9564	0.9573	0.9582	0.9591	0.9599	0.9608	0.9616	0.9625	0.9633	

90% area corresponds to a Z score of about 1.28.

0.9686 0.9706 0.9641 0.9649 0.9656 0.9664 0.9671 0.9678 0.9693 0.9699 0.9719 0.9713 0.9726 0.9732 0.9738 0.9744 0.9750 0.9756 0.9761 0.9767 0.9773 0.9778 0.9783 0.9788 0.9793 0.9798 0.9803 0.9808 0.9812 0.9817

# Probit function: the inverse

Z=1.28; convert back to raw SAT score  $\rightarrow$ 



### Statistics for Health Care

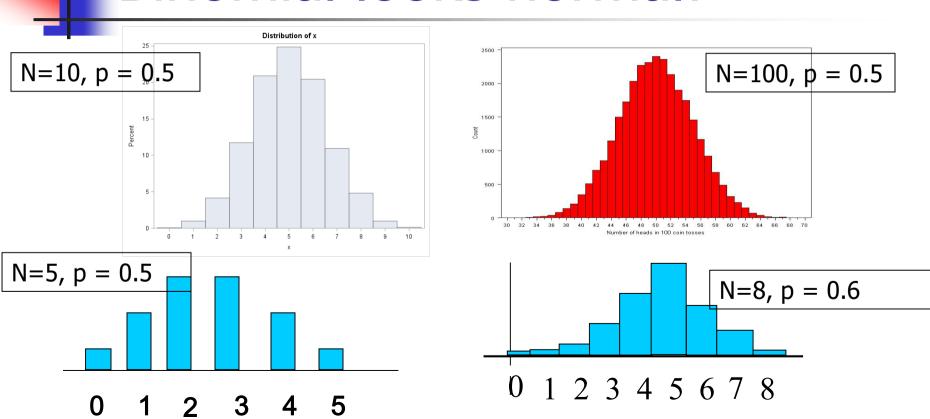
### Module 6:

The normal approximation to the binomial

# Normal approximation to the binomial

When you have a binomial distribution where the expected value is greater than 5 (np>5), then the binomial starts to look like a normal distribution...

## Binomial looks normal!



# Normal approximation to the binomial

So, we can approximate it as a normal curve with mean=np and variance = np(1-p).

# Example

You are performing a cohort study. If the probability of developing disease in the exposed group is .25 for the study duration, then if you sample (randomly) 500 exposed people, What's the probability that <u>at</u> <u>most</u> 120 people develop the disease?

## Answer

### By hand:

$$P(X \le 120) = P(X = 0) + P(X = 1) + P(X = 2) + P(X = 3) + P(X = 4) + \dots + P(X = 120) =$$

$$\binom{500}{120} (.25)^{120} (.75)^{380} + \binom{500}{2} (.25)^{2} (.75)^{498} + \binom{500}{1} (.25)^{1} (.75)^{499} + \binom{500}{0} (.25)^{0} (.75)^{500} \cdots$$

### **OR use, normal approximation**:

$$\mu = np = 500(.25) = 125$$
 and  $\sigma^2 = np(1-p) = 93.75$ ;  $\sigma = 9.68$ 

$$Z = \frac{120 - 125}{9.68} = -.52$$

$$P(Z<-.52)=.3015$$

# The binomial forms the basis of statistics on proportions...

- A proportion is just a binomial count divided by n.
  - For example, if we sample 200 cases and find 60 smokers, X=60 but the observed proportion=.30.
- Statistics for proportions are similar to binomial counts, but differ by a factor of n.



# Stats for proportions

### For binomial:

$$\sigma_x^2 = np(1-p)$$
 Differs by a factor of n.  $\sigma_x = \sqrt{np(1-p)}$ 

For proportion:

$$\mu_{\hat{p}} = p$$

$$\sigma_{\hat{p}}^2 = \frac{np(1-p)}{n^2} = \frac{p(1-p)}{n}$$

$$\sigma_{\hat{p}} = \sqrt{\frac{p(1-p)}{n}}$$

**Differs** 

factor

of n.

P-hat stands for "sample proportion."

# It all comes back to normal...

- Statistics for proportions are based on a normal distribution, because the binomial can be approximated as normal if np>5!
- If np<5, we instead use an "exact binomial" approach.



## Statistics for Health Care

### Module 7:

Assessing normality in data

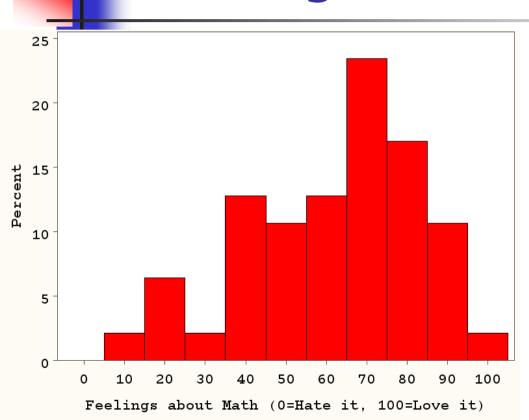
# Are my data "normal"?

- Some statistical tests assume that the data are normally distributed (especially important for small samples).
- Not all continuous data are normally distributed!
- How do you test for normality?

# Are my data normally distributed?

- 1. Look at the histogram! Does it appear bell shaped?
- 2. Look at a normal probability plot—is it approximately linear?
- 3. Look at descriptive statistcs. Are the mean and median similar? Do 2/3 of observations lie within 1 std dev of the mean? Do 95% of observations lie within 2 std dev of the mean?
- 4. Run tests of normality (such as Kolmogorov-Smirnov). But, be cautious, highly influenced by sample size!

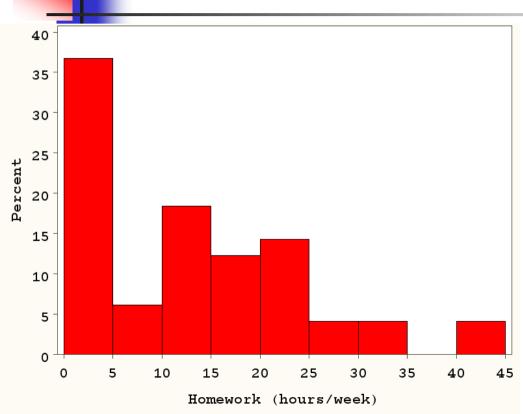
# Feelings about math...



Median = 65

Mean = 61

## Homework...



Median = 10.0Mean = 11.4