

MECOR

# Outcomes



Mama MECOR

# New York Times Oct 1, 2009

“In recent years there has been a sharp rise in reported cases of malignant melanoma, the deadliest form of skin cancer.”

- In cancer registry with 3,971 cases of melanoma, there was a 48% increase in number of cases from 1991 to 2004.
- Almost all of the increase was due to earliest stage of disease where it is difficult or impossible to distinguish between a benign & malignant lesion

# 2x2 Table

Outcome / Disease

+

-

+

Exposure

-


# Outcomes Should Be:

- Simple and feasible
- Accurate
- Reliable (perform consistently in different settings)
- Stated explicitly **before** the study starts

# How to Improve Consistency & Accuracy of Outcomes

- Standardize criteria & definitions
- Standardize methods
- Train the research staff
- Have a quality control program

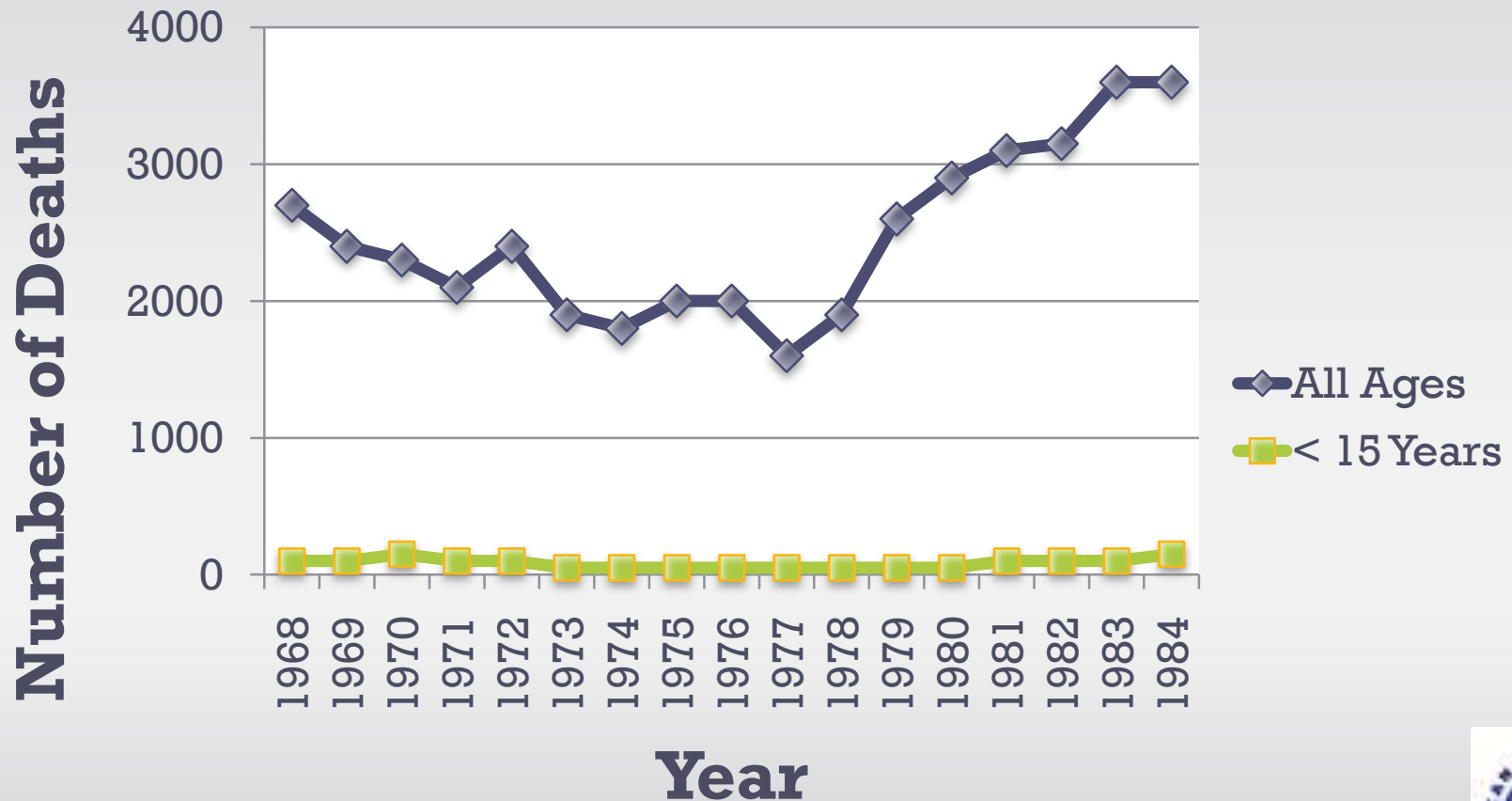
# Solutions to Problem of Defining Outcomes

- Find a standardized definition that is widely accepted
- Look in the literature for ways in which the definition has been operationalized

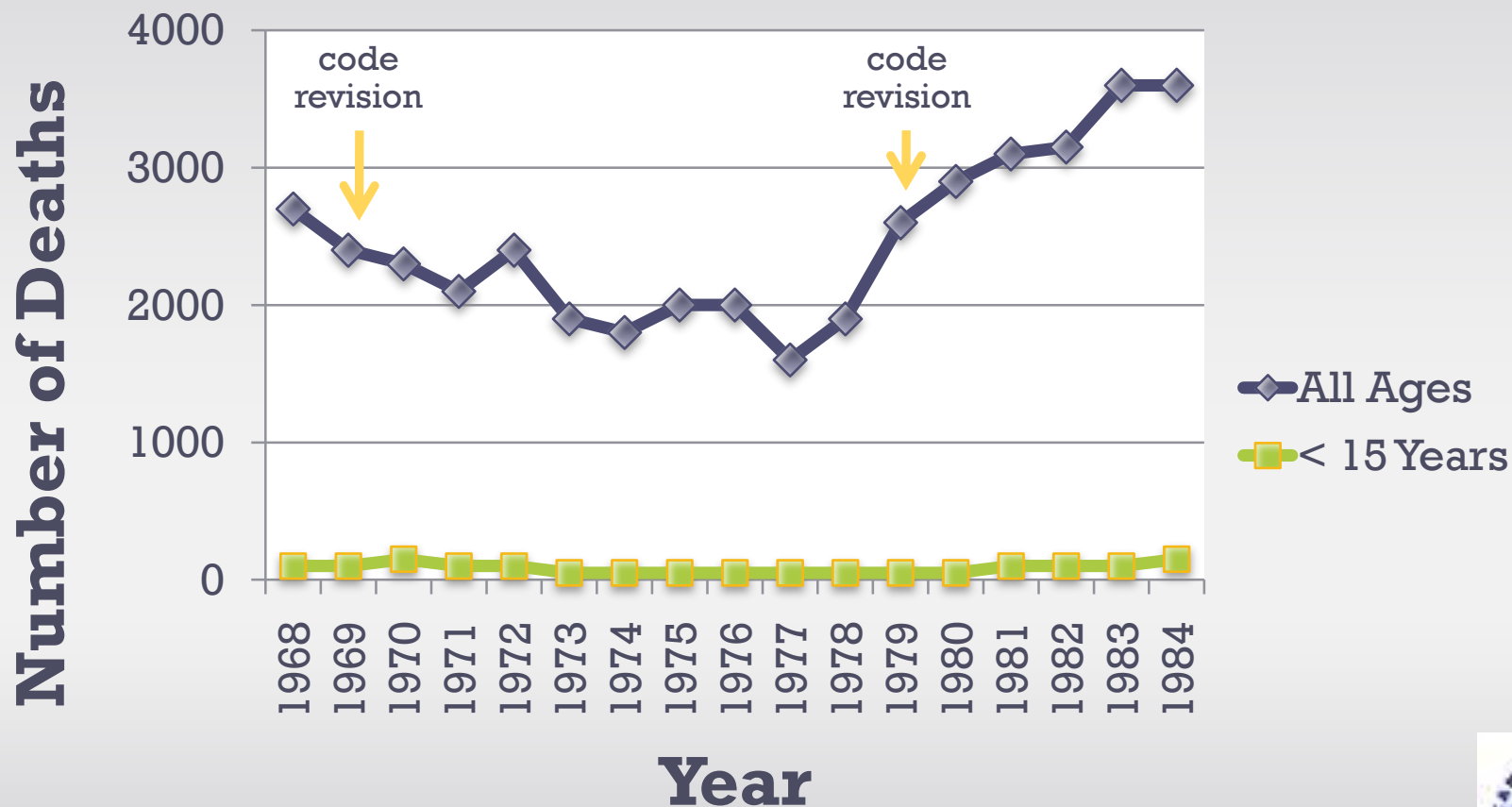
# Mortality

- A universal health outcome
- Usually comes from death certificate
- Easily available in most countries
- Classification of cause of death: ICD

# Deaths from Asthma in the U.S. by Year



# Deaths from Asthma in the U.S. by Year



# Mortality: Issues to Think About

- Death certificates are good to confirm the **fact** of death
- Variable accuracy for **cause** of death
- Accuracy varies for different diseases & countries
- Think about who completes the death certificate

# Mortality: Limitations

- Cancer if present at death is often recorded
- Chronic diseases eg COPD, diabetes, hypertension are often not
- Accuracy may vary with age & gender of person
- More than 1 disease often cause of death

# Is Mortality a Good Outcome?

Is it /can it be:

- Simple and feasible
- Accurate
- Reliable (perform consistently in different settings)
- Stated explicitly **before** the study starts

# Morbidity as an Outcome

- Doctor visits
- Other health care utilization (emergency dept visits, hospitalization)
- Respiratory symptoms
- Development of disease eg COPD, lung cancer

# Problems with Morbidity as an Outcome

- Difficulty of standardizing clinical conditions because diagnosis is often subjective
- Need for diagnostic criteria that are accepted by all

Simple, feasible

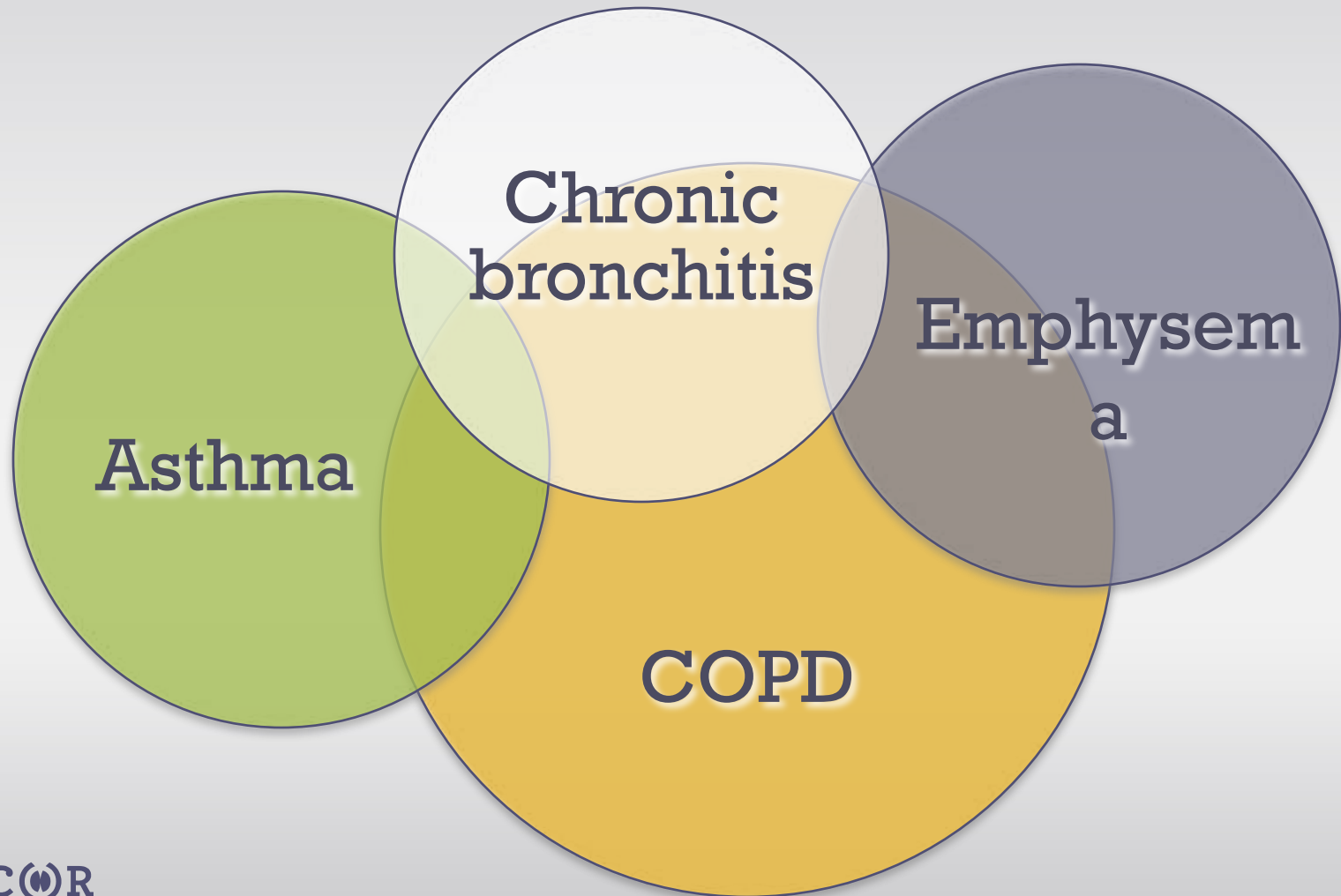
Reliable

Accurate

# Asthma or COPD

- How do you define asthma
- How do you define COPD?
- Misclassification common

# Asthma or COPD



# How to Go About Deciding on an Outcome

- Go to literature, see what others have done. Don't reinvent the wheel
- Best to focus on 1 (or a few) outcomes
- You may have to operationalize an “academic” definition

Asthma is a chronic inflammatory disorder of the airways in which many cells and cellular elements play a role: in particular, mast cells, eosinophils, neutrophils (especially in sudden onset, fatal exacerbations, occupational asthma, and patients who smoke), T lymphocytes, macrophages, and epithelial cells. In susceptible individuals, this inflammation causes recurrent episodes of coughing (particularly at night or early in the morning), wheezing, breathlessness, and chest tightness. These episodes are usually associated with widespread but variable airflow obstruction that is often reversible either spontaneously or with treatment.

— National Asthma Education & Prevention Program Guidelines, 2007  
(similar to GINA)

# Asthma as an Outcome

- Doctor diagnosis of asthma
- Wheezing in past 12 months
- History of exacerbations

- FEV1
- Response to bronchodilator
- Skin tests/RAST/Methacholine response

Simple, feasible

Reliable

Accurate

### *ISAAC Written Questionnaire (WQ)*

The ISAAC WQ on asthma comprised five questions which corresponded to the five sequences depicted in the video questionnaire. They were:

1. have you had wheezing or whistling in the chest in the last 12 months?

2. in the last 12 months, has your chest sounded wheezy during or after exercise?

3. in the last 12 months, how often, on average has your sleep been disturbed due to wheezing?

Never woken with wheezing [     ]

Less than one night per week [     ]

One or more nights per week [     ]

4. in the last 12 months, have you had a dry cough at night, apart from a cough associated with a cold or chest infection?

5. in the last 12 months, has wheezing ever been severe enough to limit your speech to only one or two words at a time between breaths?

in addition, there was a separate question of 'have you ever

# Case Definitions: Asthma

- “yes” answer to: “have you had wheezing or whistling in the chest in the past 12 months?”

## ISAAC Study

Simple, feasible | Reliable | Accurate

# Lung Function

- Which measurement should you use?  
FEV<sub>1</sub>, FEV<sub>1</sub>/FVC, IC, methacholine response & why?
- If FEV<sub>1</sub>, is this pre- or post- bronchodilator & why?

Simple, feasible

Reliable

Accurate

# Definition of COPD



- COPD is a **preventable and treatable** disease with some significant extrapulmonary effects that may contribute to the severity in individual patients
- Its pulmonary component is characterized by airflow limitation that is not fully reversible
- The airflow limitation is usually progressive and associated with an abnormal inflammatory response of the lungs to noxious particles or gases

GOLD 2006



# Diagnosis of COPD



- Diagnosis of COPD is based on a history of exposure to risk factors and the presence of airflow limitation that is not fully reversible, with or without the presence of symptoms.

GOLD 2006



# Outcome for BOLD



## Prevalence of COPD defined as:

- **Post bronchodilator** FEV<sub>1</sub>/FVC <0.7 with severity stages as defined by GOLD
- Very strict quality control program was used to standardize spirometry and how data were reviewed, cleaned and analyzed



# Classification by Severity

Stage	Characteristic
I: Mild	$FEV_1/FVC < 70\%$ ; $FEV_1 < 80\%$ predicted with or without chronic symptoms
II: Moderate	$FEV_1/FVC < 70\%$ ; $50\% < FEV_1 < 80\%$ predicted
III: Severe	$FEV_1/FVC < 70\%$ ; $30\% > FEV_1 < 50\%$ predicted
IV: Very Severe	$FEV_1/FVC < 70\%$ ; $FEV_1 < 30\%$ predicted or $FEV_1$

# Health Effects of Volcanic Ash

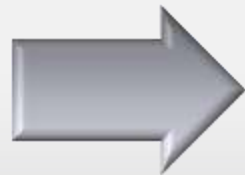
- In 1980, Mt St Helens in Washington State erupted and covered parts of Washington & Oregon in volcanic ash
- You are asked to help evaluate the health effects since people are leaving the state because of concern about the health effects & businesses are refusing to move into the state

Exposure



Outcome

Exposure to  
Volcanic Ash



Respiratory  
Eye  
Psychosocial

# Outcomes in Mt St Helens Study

- Respiratory morbidity (symptoms)
- Lung function **decline**
- Eye abrasions
- Post traumatic stress disorder
- Silicosis

Simple, feasible

Reliable

Accurate

# Decline in Lung Function

Volcanic  
Ash

+		
-		


# Shared Decision-Making

- Does engaging the patient in a shared decision making process improve outcomes?

# Shared Decision-Making



**Provider**  
Power  
Information




**Patient**  
No Power  
No Information

# Shared Decision-Making



**Provider**  
**Power**  
**Information**



**Patient**  
**Power**  
**Information**

# Overall Objective

- To improve asthma outcomes by developing and evaluating strategies for enhancing clinic-patient partnerships

# Primary Objectives

- To develop a model of shared decision-making for asthma treatment
- To evaluate the model in a 2-year randomized clinical trial in 342 adults 18–70 years with sub-optimally controlled, persistent asthma

# Primary Outcomes

- Asthma-related quality of life
- Health care utilization

Simple, feasible

Reliable

Accurate

# Secondary Outcomes

- Asthma control
- Adherence to treatment
- Symptom-free days
- Lung function
- Dispensings of asthma medications
- Satisfaction with asthma care
- Asthma-related costs
- Total asthma health care utilization

Simple, feasible

Reliable

Accurate

# Does Shared Decision Making Improve Asthma Outcomes?

		Asthma Control	
		+	-
Shared Decision Making	+		
	-		