

Different Study Designs - Pros and Cons

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Talk Outline

- Basic Design Choices
 - Observational vs. Experimental
 - Explanatory vs. Pragmatic
- Example of Pragmatic RCT

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Chinese Menu of Study Designs

BUSINESS LUNCH COMBINATIONS

Available Monday thru Friday 10:30 AM ~ 3:00 PM

All combinations served with Egg Roll and Chicken Wing OR Salad, Fried Rice,

And Choice of Hot & Sour, Wonton or Egg Drop Soup (Soup To-Go 50¢). Add a Side Salad for 50¢.

1	Assorted Vegetable Chicken	5.60	28	Mongolian Beef	5.99
2	Cashew Chicken	5.60	29	Assorted Vegetable Beef	5.99
3	Broccoli Chicken	5.60	30	Shredded Beef Szechuan Style (hot)	5.99
4	Kung Pao Chicken (hot)	5.60	31	Spicy Beef in Garlic Sauce (hot)	5.99
5	Asparagus Chicken	5.95	32	Hunan Beef (hot)	5.99
6	Fresh Mushroom Chicken	5.60	32A	Spicy Basil Beef (hot)	5.99
7	Mongolian Chicken	5.60	33	Double Mongolian (Chicken & Beef)	5.99
8	Sweet & Sour Chicken	5.60	33A	Triple Mongolian	6.50
9	Sesame Chicken	5.60		(Chicken, jumbo shrimp and beef)	
10	General Tso's Chicken (hot)	5.60	33B	Triple Teriyaki (Chk, Shrimp & Beef)	6.50
11	Almond Fried Chicken	5.60	34	Twice Cooked Pork (hot)	5.60
12	Teriyaki Chicken	5.60	35	Sweet & Sour Pork	5.60
13	Spicy Chicken in Garlic Sauce (hot)	5.60	36	Spicy Pork in Garlic Sauce (hot)	5.60
14	Curry Chicken (hot)	5.60	37	Kung Pao Three Ingredients (hot)	6.50
15	Hunan Chicken (hot)	5.60		(Chicken, jumbo shrimp and beef)	
16	Black Pepper Chicken	5.60	38	Happy Family	6.95
17	Hunan Chicken & Shrimp (hot)	5.95		(Scallop, shrimp, Chicken, pork & beef)	
18	Braised Shrimp in Szechuan Sauce (hot)	6.25	40	House Special Fried Rice	5.60
19	Kung Pao Shrimp (hot)	6.25		Also available in chicken, beef, pork, shrimp or vegetable	
20	Sweet & Sour Shrimp	5.95	41	House Special Lo Mein	5.75
21	Asparagus Shrimp	6.50		Also available in chicken, beef, pork, shrimp or vegetable	
22	Shrimp with Lobster Sauce	6.25	43	Stir-fried Assorted Vegetables	5.60
23	Fresh Broccoli Shrimp	6.25	44	Hot & Spicy String Beans (hot)	5.60
24	Teriyaki Shrimp	6.25	45	Stir-Fried Mixed Vegetables with Tofu	5.75
25	Hunan Shrimp (hot)	6.25	46	Steamed Mixed Vegetables	5.60
26	Green Pepper Steak	5.99	47	Steamed Veggies with Chicken	5.60
27	Fresh Broccoli Beef	5.99	48	Steamed Veggies with Shrimp	5.95

Menu of Study Designs

Column A

- Observational
 - Case series
 - Cross-sectional
 - Case-control
 - Cohort
- Experimental
 - RCT

Column B

- Explanatory
- Pragmatic

Case Series

- Observational
- Retrospective
- Descriptive, not analytic
- Hypothesis generating
- No comparison group

Cross-sectional Study

- Group examined at 1 point in time
- Observational
- Retrospective/prospective
- Usually descriptive
- Usually the design of diagnostic accuracy studies

Case-Control

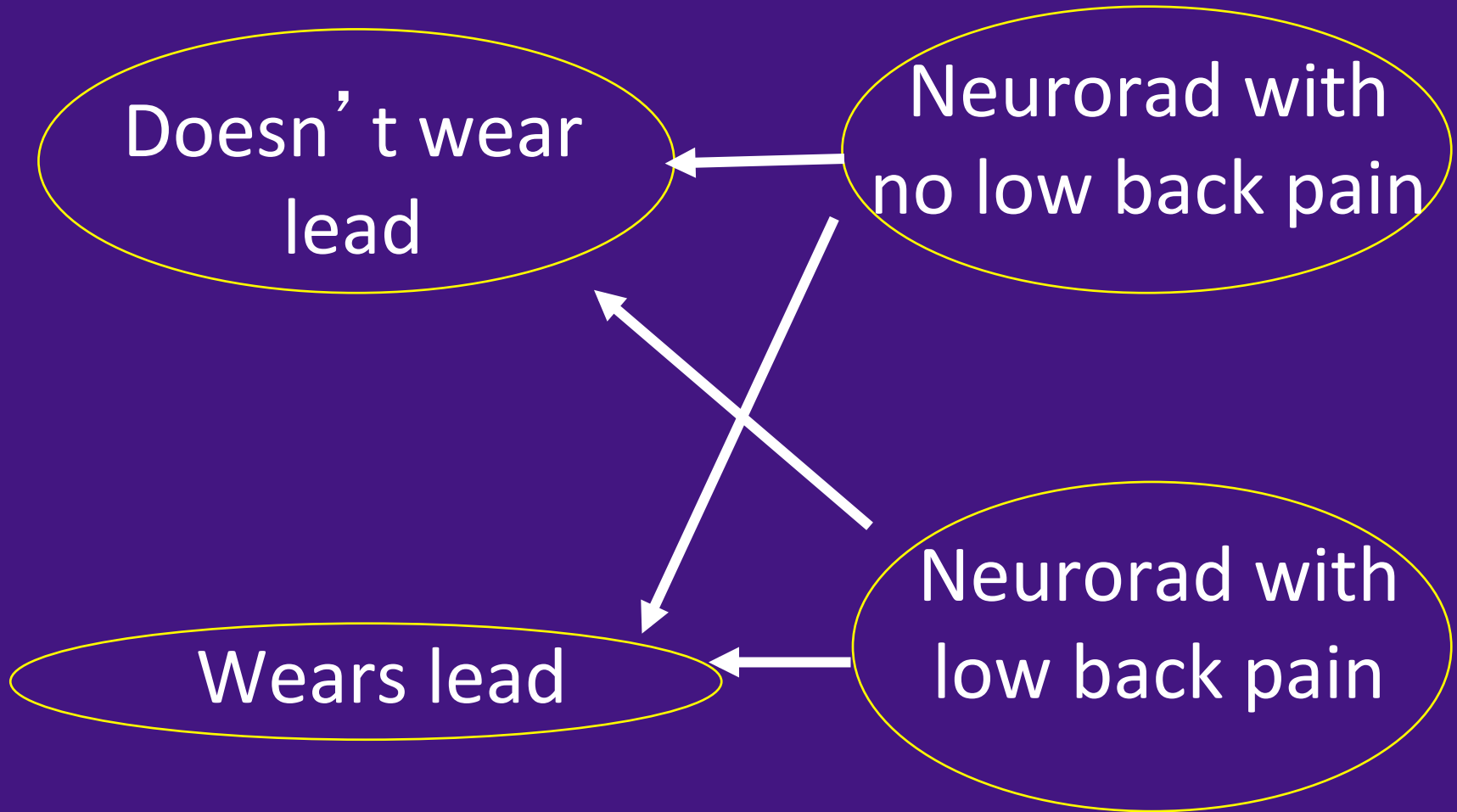
- 2 groups defined by outcome
- Observational
- Retrospective- outcomes need to have occurred
- Descriptive or analytic
- Good for rare outcomes

Study Question: Is
Wearing Lead Associated
with Low Back Pain in
Neuroradiologists?

Case Control

Risk factor

Outcome



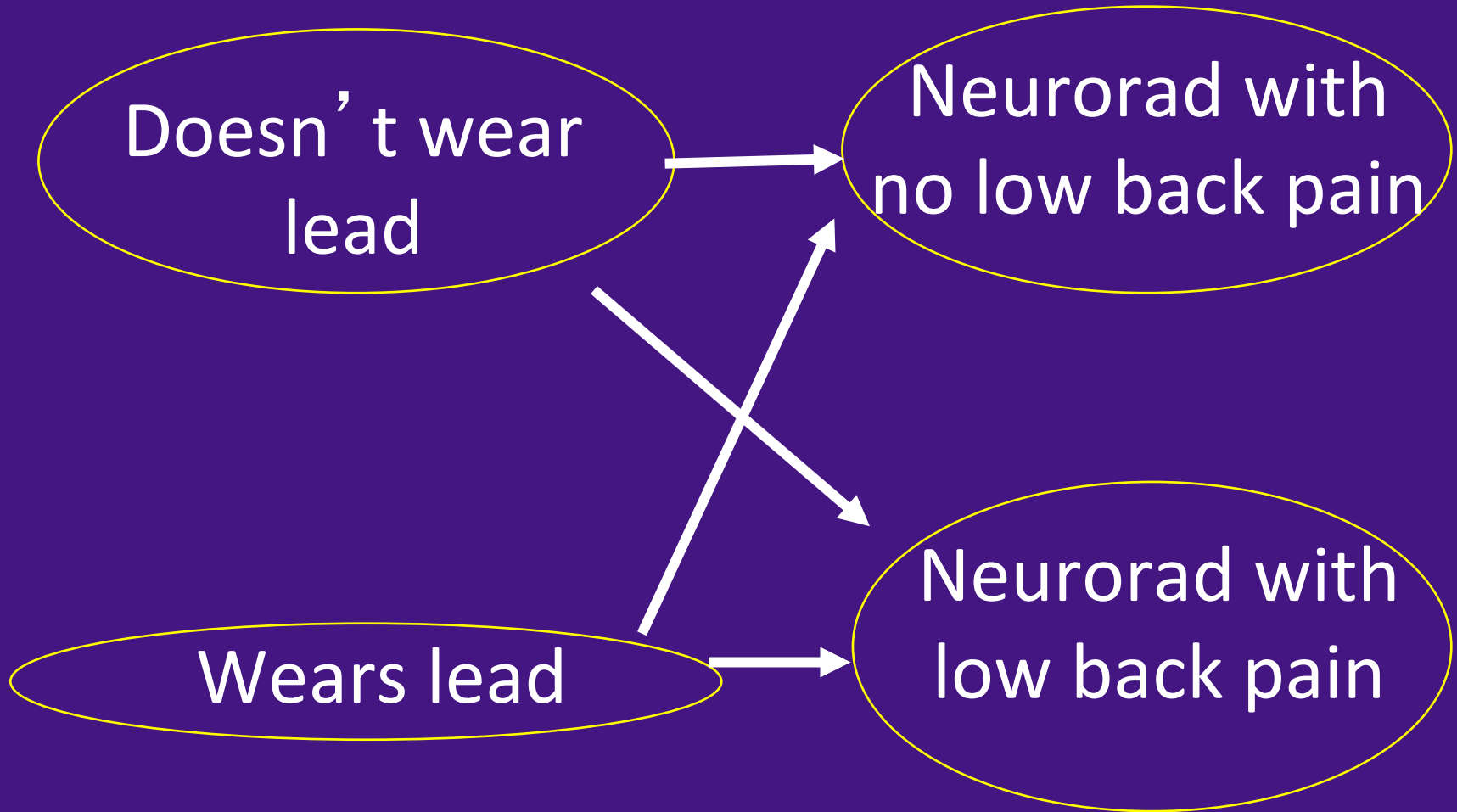
Cohort Study

- 2 groups defined by risk factor
- Observational
- Prospective or retrospective
- Descriptive or analytic
- Group followed over time
- Good for rare risk factors

Cohort Control

Risk factor

Outcome



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Randomized Controlled Trials (RCTs)

- Experiment
- Groups created randomly
- Prospective
- Analytic
- Best design for eliminating bias

RCTs

- Only design that controls for unknown biases

RCTs

- Only design that controls for unknown biases
- Cohort and case-control designs can control for known biases

Picking an item not the end of choices...

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Gluten Free

Nut free

Veggie

Some RCT Choices- Unit of Randomization

- Individual subject/participant
- Cluster (consider if contamination an issue)
 - Provider
 - Clinic
 - Health system

Some RCT Choices- Type of Randomization

- Parallel
- Cross-over
- Stepped-wedge

Some RCT Choices- Type of Randomization

	<u>Parallel</u>
	Time
	1
Cluster	1
	1
	0
	0

Some RCT Choices- Type of Randomization

		<u>Parallel</u>			<u>Crossover</u>
		Time			Time
		1			1 2
Cluster	1	1	Cluster	1	1 0
	2	1		2	1 0
	3	0		3	0 1
	4	0		4	0 1

Some RCT Choices- Type of Randomization

M.A. Hussey, J.P. Hughes / Contemporary Clinical Trials 28 (2007) 182–191

		<u>Parallel</u>					<u>Crossover</u>		<u>Stepped Wedge</u>				
		Time					Time		Time				
		1					1 2		1 2 3 4 5				
Cluster	1	1		1	0		1	0	1	1	1	1	1
	2	1		1	0		2	0	0	1	1	1	
	3	0		0	1		3	0	0	0	1	1	
	4	0		0	1		4	0	0	0	0	0	1

Stepped Wedge vs. Parallel Design

- Controls for external temporal trends
- More efficient than parallel design since have both between and within group comparisons
- Assures all sites receive intervention
→ Participation more palatable for interventions viewed as desirable
- Conduct and analysis more complex

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Pragmatic vs. Explanatory Trials

CMAJ

ANALYSIS

A pragmatic–explanatory continuum indicator summary (PRECIS): a tool to help trial designers

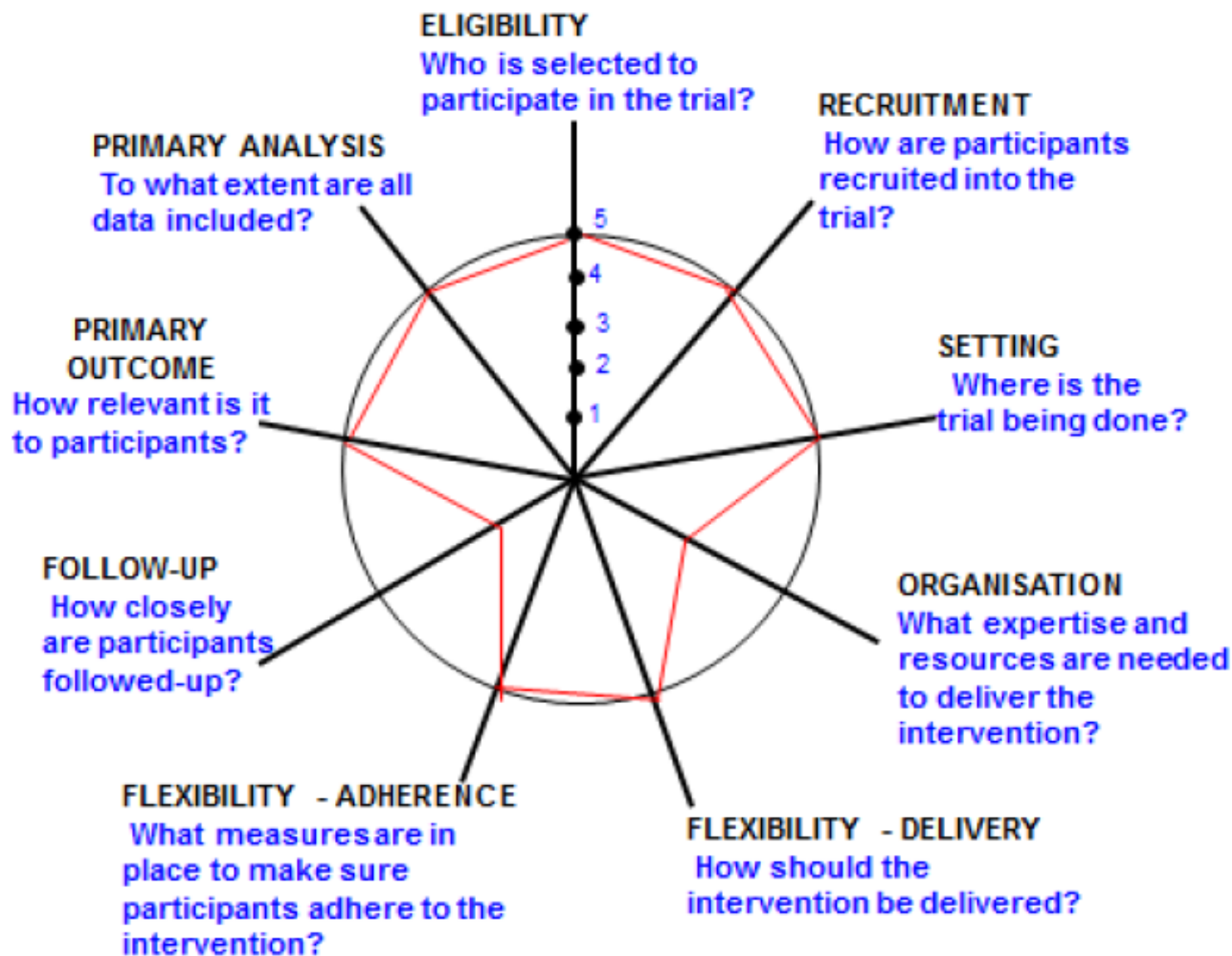
Kevin E. Thorpe MMath, Merrick Zwarenstein MD MSc, Andrew D. Oxman MD, Shaun Treweek BSc PhD, Curt D. Furberg MD PhD, Douglas G. Altman DSc, Sean Tunis MD MSc, Eduardo Bergel PhD, Ian Harvey MB PhD, David J. Magid MD MPH, Kalipso Chalkidou MD PhD

Published at www.cmaj.ca on Apr. 16, 2009. An abridged version of this article appeared in the May 12 issue of *CMAJ*. This article was published simultaneously in the May 2009 issue of the *Journal of Clinical Epidemiology* (www.jclinepi.com).

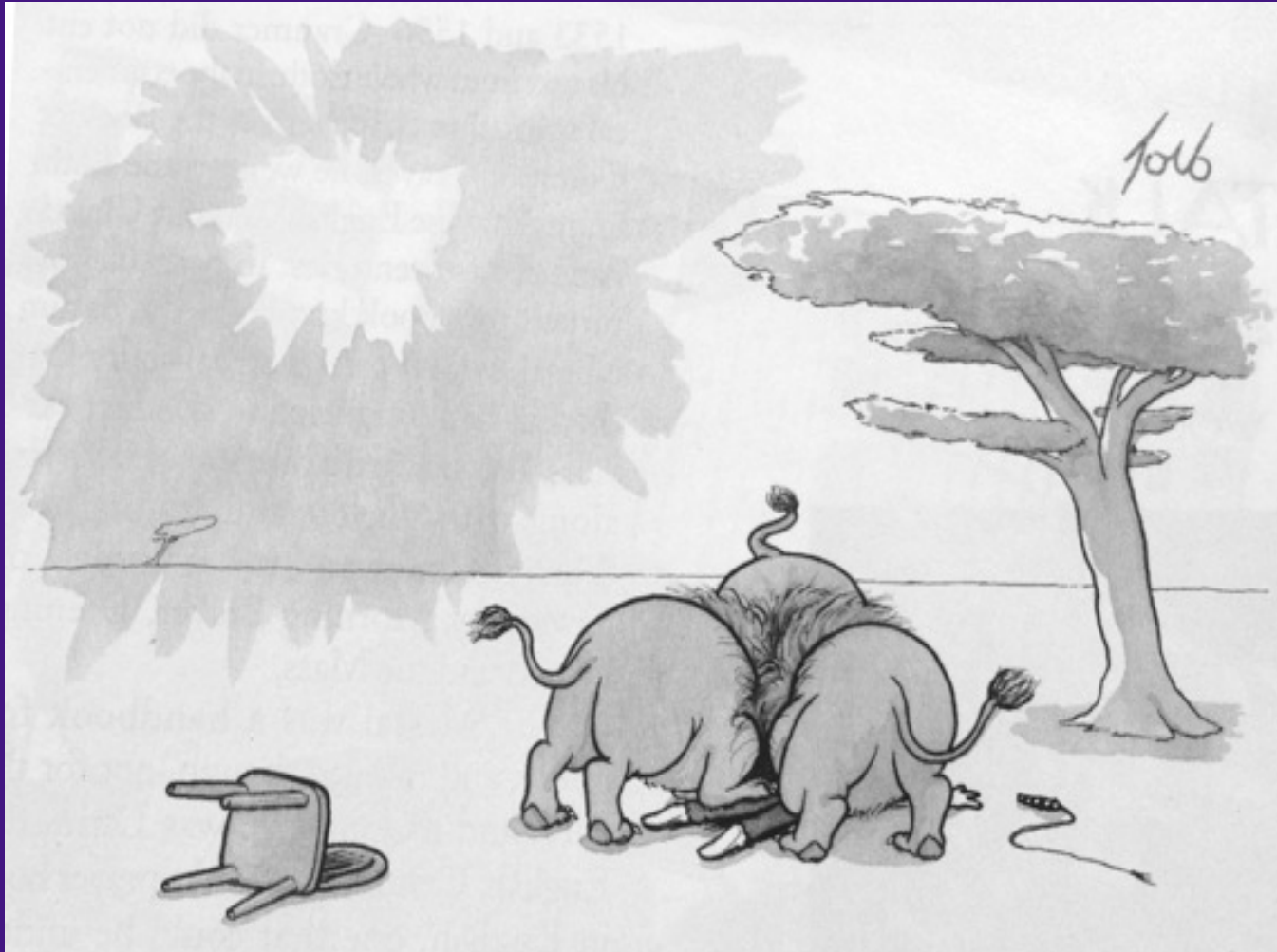
Pragmatic vs. Explanatory Trial

- Explanatory trials
 - Examine efficacy
 - Conducted under ideal conditions
 - Explain mechanisms
- Pragmatic trials
 - Determine comparative effectiveness (CER)
 - Routine practice
 - Aim to help providers, patients, and policy makers choose between interventions

Example from: Little P, Moore M, Kelly J, Williamson I, Leydon G, McDermott L, Mullee M, Stuart B: Ibuprofen, paracetamol, and steam for patients with respiratory tract infections in primary care: pragmatic randomised factorial trial. BMJ 2013, 347:f6041.



Explanatory vs. Pragmatic Trials



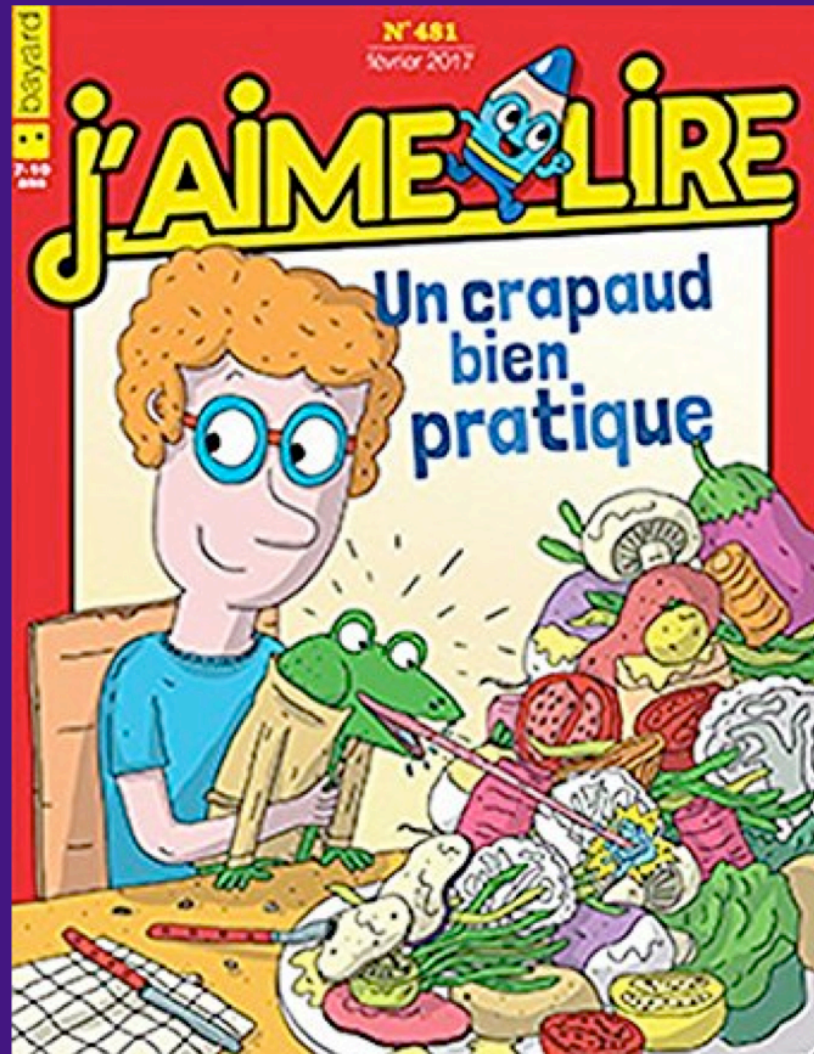
The Great Zeferelli's chair worked a lot better
in controlled conditions.

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Example of Pragmatic Trial- Lumbar Imaging with Reporting of Epidemiology (LIRE)

LIRE (pronounced *leer*)
from the French verb, 'to read'.



rethinkingclinicaltrials.org



NIH COLLABORATORY
LIVING TEXTBOOK
of Pragmatic Clinical Trials



HOME WELCOME GRAND ROUNDS NEWS



DESIGN



CONDUCT

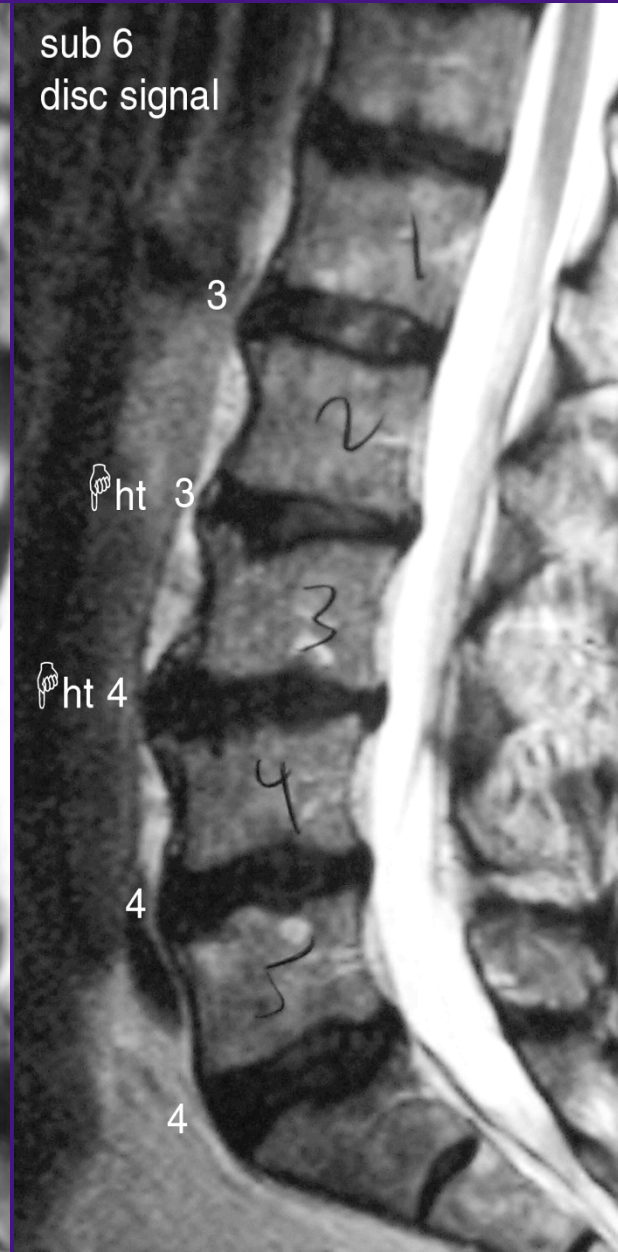
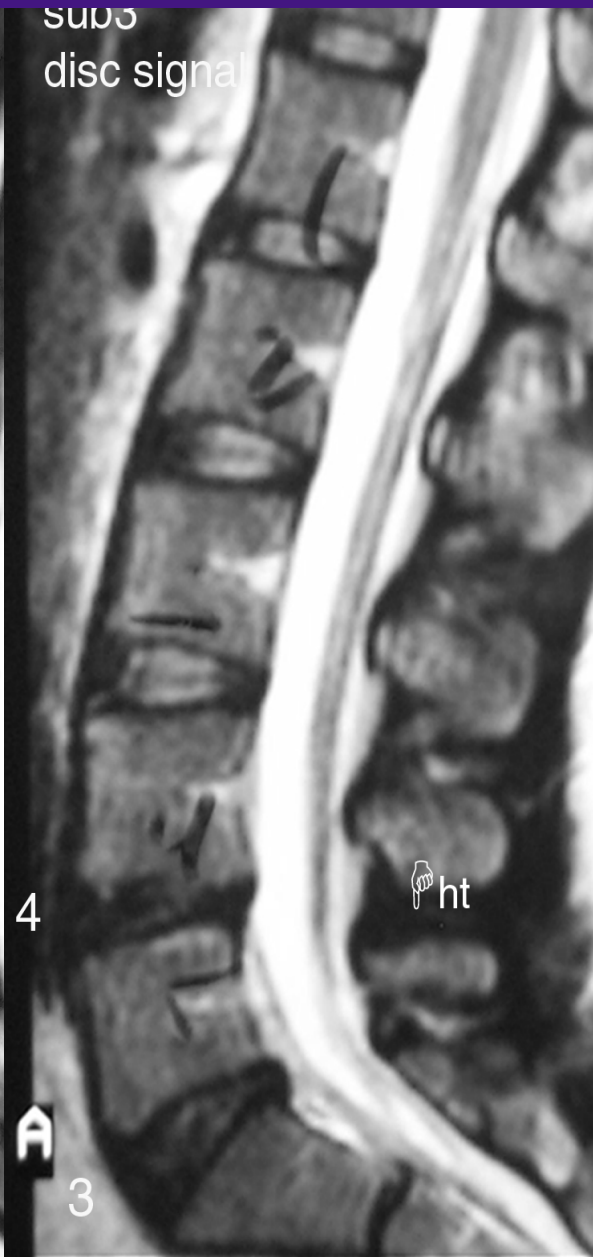


DISSEMINATION

Background and Rationale

- Lumbar spine imaging frequently reveals incidental findings
- These findings may have an adverse effect on:
 - Subsequent healthcare utilization
 - Patient health related quality of life

Disc Degeneration in Asx



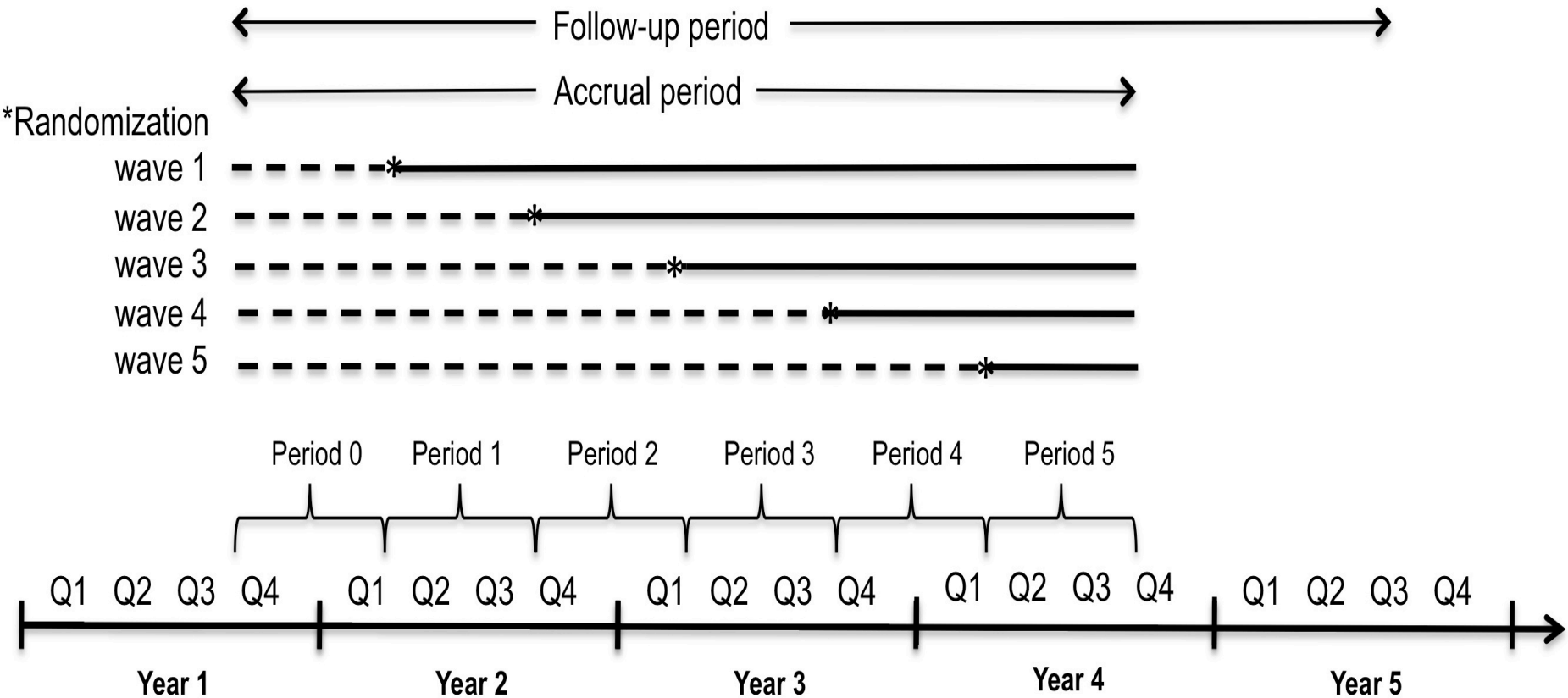
Participating Systems

Name	# Primary Care Clinics (Randomized)	# PCPs (Randomized)
Kaiser Perm. N. California	21	2,349
Henry Ford Health System, MI	26	187
Kaiser Permanente of Washington	19	365
Mayo Health System	34	400
Total	100	3,301

Stepped Wedge RCT

— Exposed to LIRE intervention

- - - Unexposed to LIRE intervention



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"I'm afraid you've had a paradigm shift."