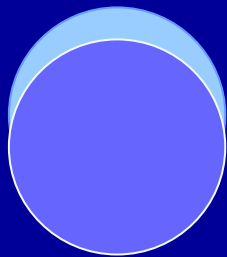


Establishing an Investigator Initiated Trial

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University of Melbourne



**MURDOCH CHILDRENS
RESEARCH INSTITUTE**



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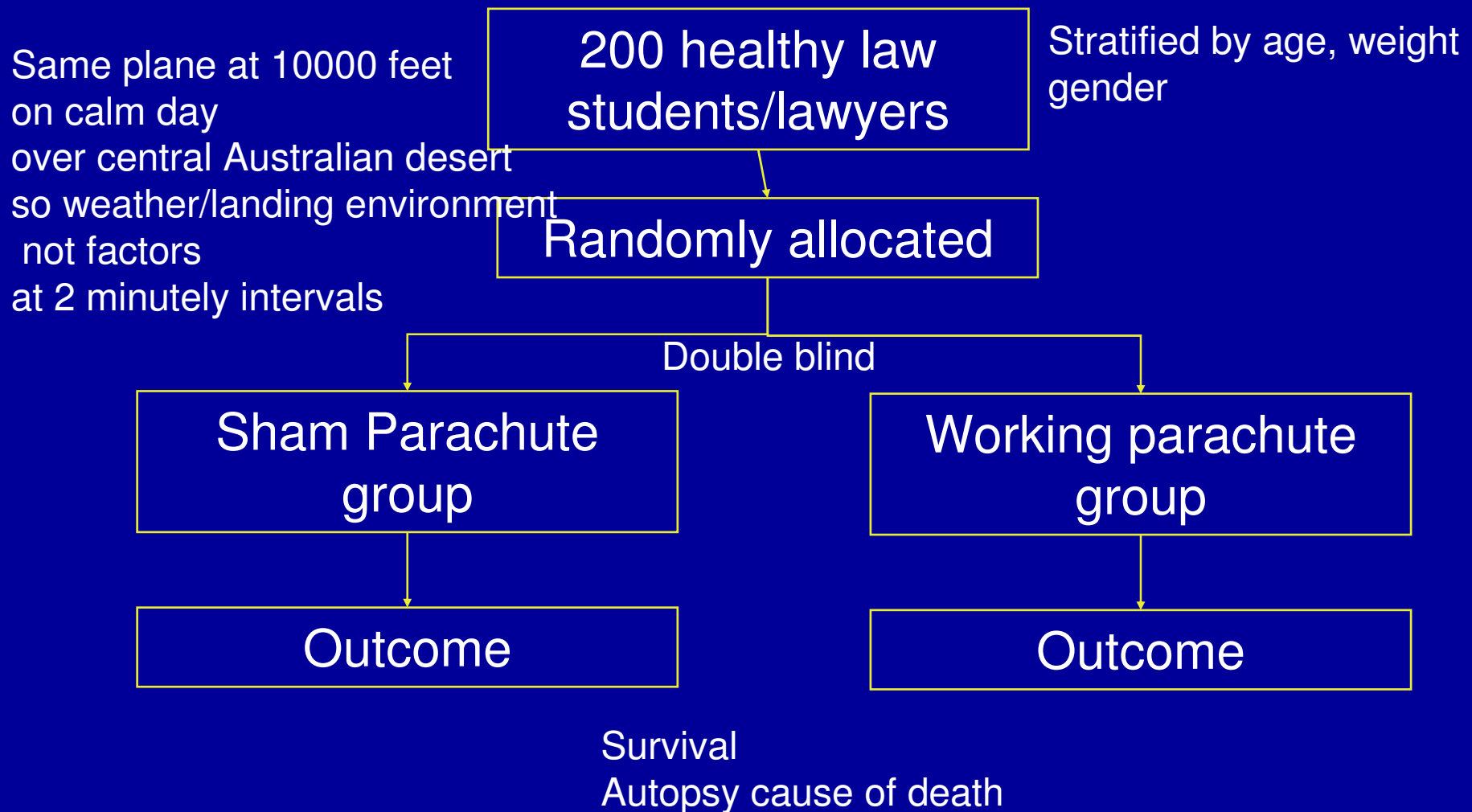
**ROYAL
CHILDREN'S
HOSPITAL**



News Headline 19th May 2009

“British man falls from 6000 feet and forgets to open his parachute. He is now resting at home with his wife”

The wopflop trial: RCT of Working Open Parachutes versus Failure to Open Parachutes



If I can't get funding for that:

- Some people complain that not getting paid on time makes them unhappy
- But there are many confounders and people could be unhappy for lots of reasons
- We need a randomised trial to justify increasing resources in pay office so people can be paid on time

The SPIT trial: RCT of Staff Paid Incorrectly versus on Time trial

Need a >50% difference in outcome to justify improving the service

1000 employees

Inclusion criteria:
all full/part time staff (stratified)
Exclusion criteria:
Head of department haematology

Randomly allocated

Paid correctly but late

($10 < x < 14$ days)

Paid correctly and on time

QoL measure

Work satisfaction rating

QoL measure

Work satisfaction rating

Masters of Health Research Methodology McMaster University

- CHS-HRM 721 Fundamentals of health research and evaluation methods.
- CHS-HRM 702 Introduction to biostatistics.
- CHS-HRM 730 Research methods for randomised controlled trials.
- CHS-HRM 733 Statistical and methodological issues in controlled clinical trials.
- CHS-HRM 734 Data management in health research.
- CHS-HRM 751 Observational and analytical research methods.
- Major Thesis : *A randomised trial of prophylactic anticoagulation in children following Fontan surgery*

CHS-HRM 799

Acronyms for clinical trials

- Optimal acronym length
- Use of numbers for subsequent trials
- The value of rhyming syllables
- How to avoid offending religious or ethnic groups
- Acronyms for any language
- How much is too much innuendo
- Subliminal messages in trial acronyms
- How to design trial questions and budgets to fit your acronym

Is it all in the name ?

Long term prophylaxis after acute coronary syndromes

- AFTER
 - Aspirin/Anticoagulants Following Thrombolysis with Anistreplase (Eminase) and Recurrent Infarction
- CARS
 - Coumadin aspirin reinfarction study
- CHAMP
 - Combined Hemotherapy and Mortality Prevention study

Is it all in the name ?

Long term prophylaxis after acute coronary syndromes

- OASIS
 - Organization to Assess Strategies for Ischemic Syndromes
- APRICOT
 - Antithrombotics in the Prevention of Reocclusion in Coronary Thrombolysis
- TIMI, ASPECT, WARIS

The question is the key element



How do you develop the question?

- Clinical service
 - Post Fontan children on warfarin
- Discussion with colleagues
 - Initial informal chats
 - Survey through national groups
 - Understand their priorities, concerns
- Review of literature
 - Comprehensive systematic review
 - Publish in a journal your colleagues will read

Developing the question

- Must be in equipoise
 - Based on the evidence
- Must be ethical based on current standard of practice
 - Especially in considering placebo groups
- Must be clear and specific and capable of being answered

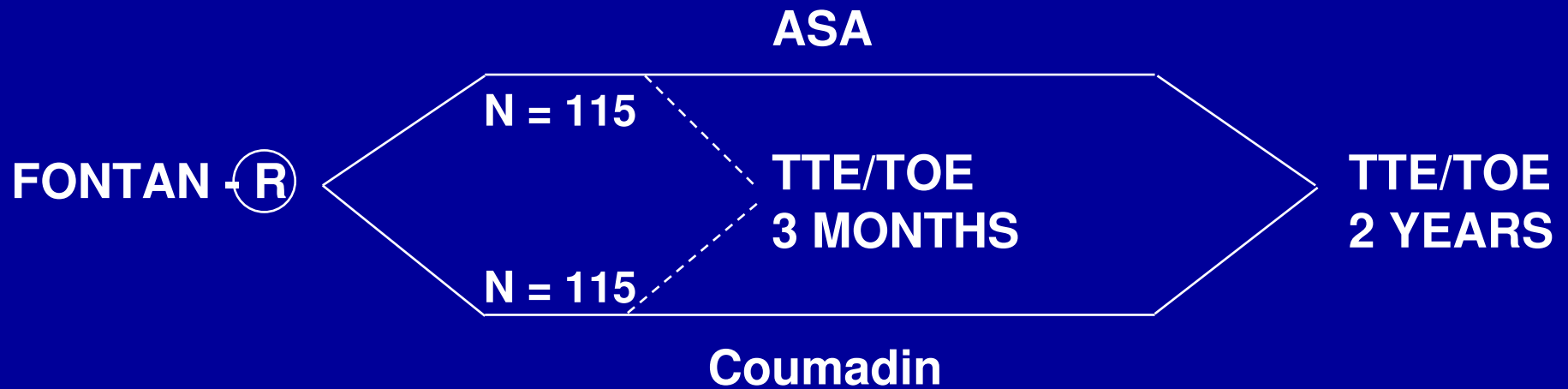
Developing the protocol

- Clinical service
 - Consider standard practice in terms of review visits, investigations, etc
- Discussion with colleagues
 - Workshops
 - Circulating Drafts and key questions
 - Protocolize every step
- Review of literature
 - Continue to keep up to date
 - Support each step of the trial especially outcome measures

Developing the protocol

- Need to make your colleagues have ownership- a commitment to the cause
 - communication, communication and more communication
- Need to have patients on side
 - Use parent support groups
- Need to have expertise/experience
 - Getting from idea to implementable study is not easy.

FONTAN A



Heart & Stroke Foundation of Canada
NHF Australia

**UNDERSTAND AND BE ABLE TO
EFFECTIVELY PREVENT/TREAT
THROMBOSIS IN CHILDREN**

**APPLY KNOWLEDGE TO
ADULTS & OTHER DISEASES**

**CLINICAL
TRIALS
IN CHILDREN**

**BROADER
IMPLICATIONS
OF
DEVELOPMENTAL
HAEMOSTASIS**

**LONG TERM
OUTCOMES**

**THERAPEUTIC
MONITORING**

**PHARMACOLOGY
OF
THERAPEUTIC
AGENTS**

**BASIC
EPIDEMIOLOGY**

**DIAGNOSTIC
STRATEGIES
& DISEASE
MONITORING**

**DEVELOPMENTAL
HAEMOSTASIS**

Clinical thrombosis and anticoagulation service

Be determined- never give up

