

MAKING PRAGMATIC TRIALS WORK

identification of operational challenges &
development of a decision support tool for pragmatic trial design

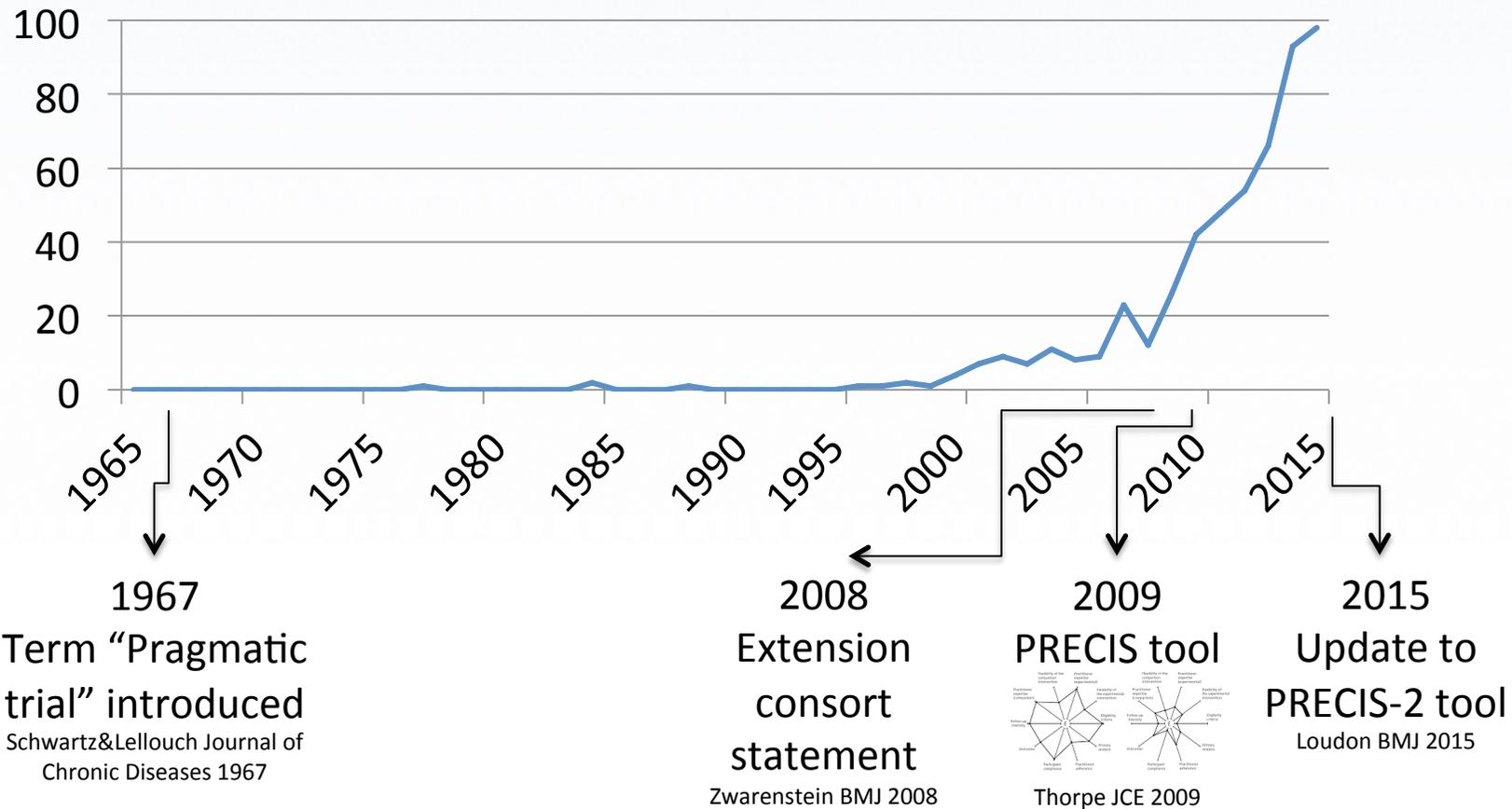
WP3

Iris Goetz, Mira Zuidgeest

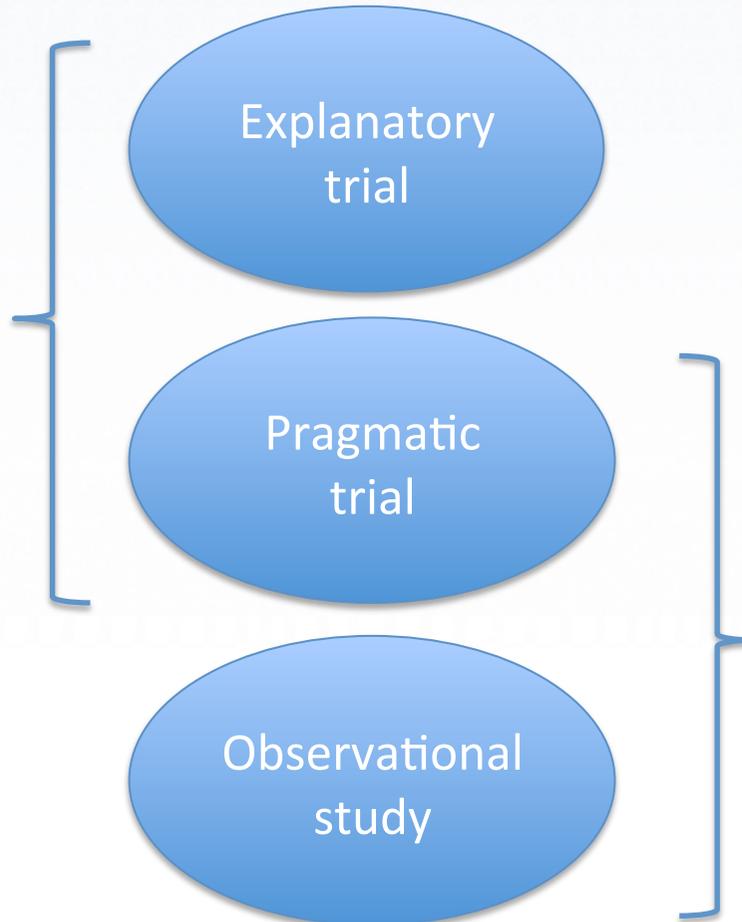
November 24, 2016



Number of PubMed hits with 'pragmatic' and 'trial' in title



Randomization
=
prognostic comparability between patient groups



Real World Evidence
=
How does treatment strategy A compare to treatment strategy B (often usual care) in daily clinical practice?

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Effectiveness of Fluticasone Furoate– Vilanterol for COPD in Clinical Practice

Jørgen Vestbo, D.M.Sc., David Leather, M.B., Ch.B., Nawar Diar Bakerly, M.D.,
John New, M.B., B.S., J. Martin Gibson, Ph.D., Sheila McCorkindale, M.B., Ch.B.,
Susan Collier, M.B., Ch.B., Jodie Crawford, M.Sc., Lucy Frith, M.Sc.,
Catherine Harvey, D.Phil., Henrik Svedsater, Ph.D., and Ashley Woodcock, M.D.,
for the Salford Lung Study Investigators*

N ENGL J MED 375;13 NEJM.ORG SEPTEMBER 29, 2016



The research leading to these results has received support from the Innovative Medicines Initiative Joint Undertaking under grant agreement no [115303], resources of which are composed of financial contribution from the European Union's Seventh Framework Programme (FP7/2007-2013) and EFPIA companies' in kind contribution.
www.imi.europa.eu

Design choices

Operational challenges

Pre-launch

Timely safety reporting

Treatment strategy with once-daily inhaler

Real world drug supply

Broad patient inclusion in GP practices

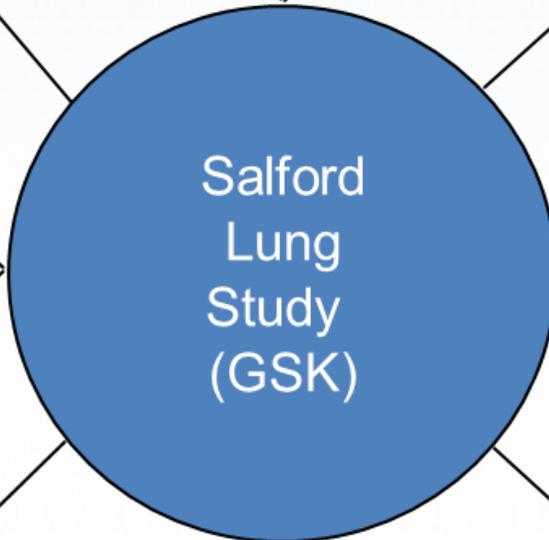
Informed consent

Usual care comparator

GCP training

Clinically relevant outcome COPD exacerbation

Linkage of EHR



BUT! Generalisability beyond Salford?

Nawar 2013; New 2014



Pragmatic trials



opportunity to generate RWE in early stage of drug development



limited experience & unanticipated operational challenges

WP3

- Identify operational challenges 
- Analyse impact on generalizability, validity, precision, stakeholder & ethical acceptability, resource use 
- Identify and propose solutions 
- Overview paper series 
- Develop decision support tool for pragmatic trial design **PragMagic** 

Name of trial
email@something.nl

PRAGMAGIC ILLUMINATE THE CITY ⓘ

- Most optimal
- Opportunity for improvement
- More opportunity for improvement
- No implication

Trial information
Options
Report

PRAGMAGIC
a decision support tool for
pragmatic trial design

Total 55%

Operational challenges 2
Notes 2

Quality Site
Safety Participant
Data Comparator
Outcome

Retropro & eLung
Van Staa 2014



59%
of sites expressed
interest in trial
participation

4%
of sites actually
recruited patients

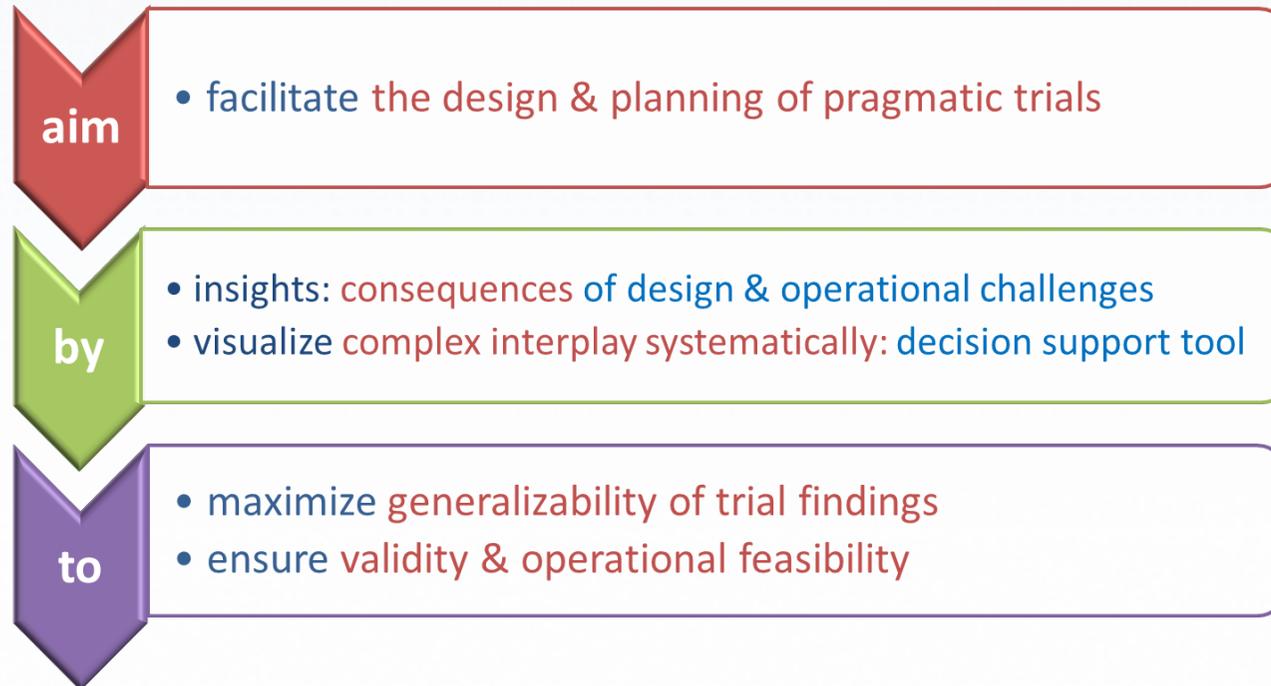
generalizability ↓
& feasibility ↓

participating GPs differed from those not participating

eLung trial did not recruit sufficient N of patients

- [03Brussel-GetReal-Pragmagic-klikmodel.mov](#)





What PragMagic is NOT



- NOT a decision-making tool;
- NOT a checklist to assure (regulatory/ethical) compliance;
- NOT a quality check/verdict on study design.

Watch this space:

WWW.PRAGMAGIC.EU

first version of tool
available early 2017



Run through a “touch-and-feel” version of
PragMagic 12:00h-13:15h this afternoon!