MAKING PRAGMATIC TRIALS WORK

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

WP3

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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.

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Pragmatic trials as a solution for RWE need

Randomization
= prognostic comparability between patient groups

Explanatory trial

Pragmatic trial

Observational study

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

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### What are pragmatic trials?

Pragmatic trials are designed to be generalisable to real-world settings and populations. They aim to answer the question of whether a treatment works in real-world conditions.

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<th>Continuum</th>
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<td><strong>WHAT?</strong></td>
<td><strong>Generalisable to real world population</strong></td>
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<td><strong>Hypothesis testing:</strong> Assess cause – effect of drug</td>
<td><strong>WHY?</strong></td>
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<td><strong>Minimize variation:</strong> Rigid protocol, ideal circumstances</td>
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<td><strong>Important to decision/policy makers &amp; patients</strong></td>
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<td>- Selective inclusion of participants &amp; sites</td>
<td><strong>WHO?</strong></td>
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<td>- Compare drugs or against placebo</td>
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<td>- Outcomes research relevant</td>
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<td>- Data collection &amp; monitoring &gt; usual practice</td>
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**WHAT?**
- Do treatment work?

**WHY?**
- Does treatment work?

**HOW?**
- Minimize variation: Rigid protocol, ideal circumstances

**WHO?**
- Selective inclusion of participants & sites

**METHOD?**
- Compare drugs or against placebo
- Outcomes research relevant
- Data collection & monitoring > usual practice
Effectiveness of Fluticasone Furoate–Vilanterol for COPD in Clinical Practice


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Design choices

Pre-launch

Treatment strategy with once-daily inhaler

Broad patient inclusion in GP practices

Usual care comparator

Clinically relevant outcome COPD exacerbation

Operational challenges

Timely safety reporting

Real world drug supply

Informed consent

GCP training

Linkage of EHR

Salford Lung Study (GSK)

BUT! Generalisability beyond Salford?

Nawar 2013; New 2014
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PRAGMAGIC
a decision support tool for pragmatic trial design
Forces at work

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
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• 03Brussel-GetReal-Pragmagic-klikmodel.mov
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The goal of PragMagic

aim

• facilitate the design & planning of pragmatic trials

by

• insights: consequences of design & operational challenges
• visualize complex interplay systematically: decision support tool

to

• maximize generalizability of trial findings
• ensure validity & operational feasibility

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!