From efficacy to effectiveness: the Salford Lung Study

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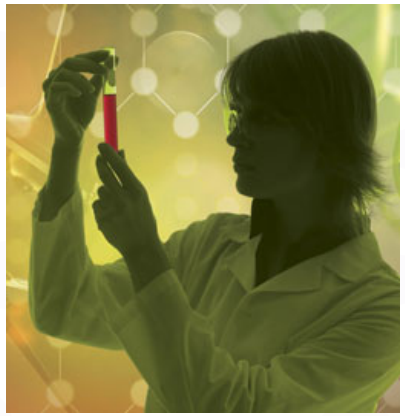
Evidence needed for medicines in today's world



Efficacy

Risk / Benefit

Effectiveness

Value for money

Individual and Community

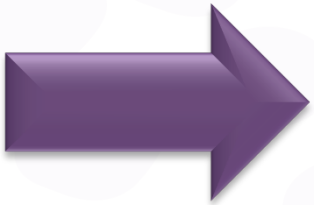
Efficacy to Effectiveness

Gold standard science to answer specific questions

Evidence representing medicines in the real world

Randomised Controlled Trials

Double blind
Double dummy
Strict inclusion criteria
Exclusions
Adherence encouraged
Frequent reviews
Drugs provided
Traditional Efficacy Endpoints



Effectiveness

Open label
Broad population
All comers
Set in normal care
No extra review
Drugs prescribed and collected in usual way
Health Outcome and Utilisation Endpoints
i.e. Real life

Salford Lung Study Ambition

Study is as near to usual care ("real world") as possible using a pre-license medicine

- **embrace heterogeneity of patient population**
- **normalise the patient experience as much as possible**
- **pragmatic – "usual care" in each arm**
- **relevant endpoints collected**

Maintain Scientific Rigour

- **Interventional**
- **Randomised**
- **Controlled**

Study outline for COPD

Primary endpoint: Moderate/severe exacerbation (defined by oral steroid (and/or antibiotic use) +/- hospitalisations)
Secondary endpoints: Serious Pneumonias, Healthcare utilisation, COPD Assessment Test (CAT)

2800 patients

- **Patients in primary care, aged 40+**
- **GP diagnosis of COPD**
- **Taking ICS, LABA, LAMA alone or in combination**
- **Exacerbation in last 3 years**
- **Consented**

Randomised

New Rx open label

Visit 2
Routine respiratory review
Device instruction
CAT

12 months of usual care

Visit 6
Routine respiratory review
CAT

Existing maintenance Rx, ICS, LABA, LAMA

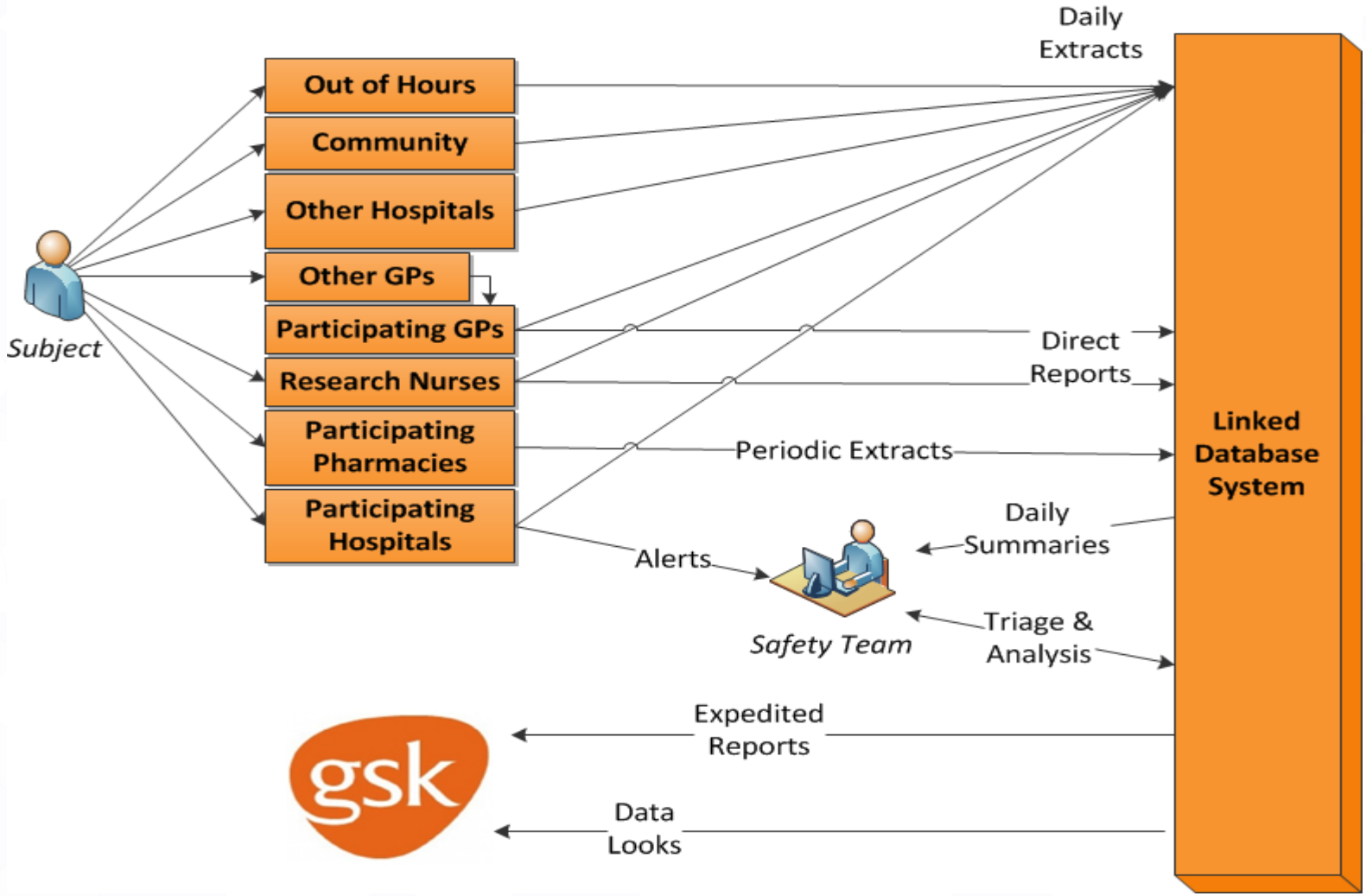
Constant real-time data collection of all HC interventions/safety monitoring

Challenges and Solutions

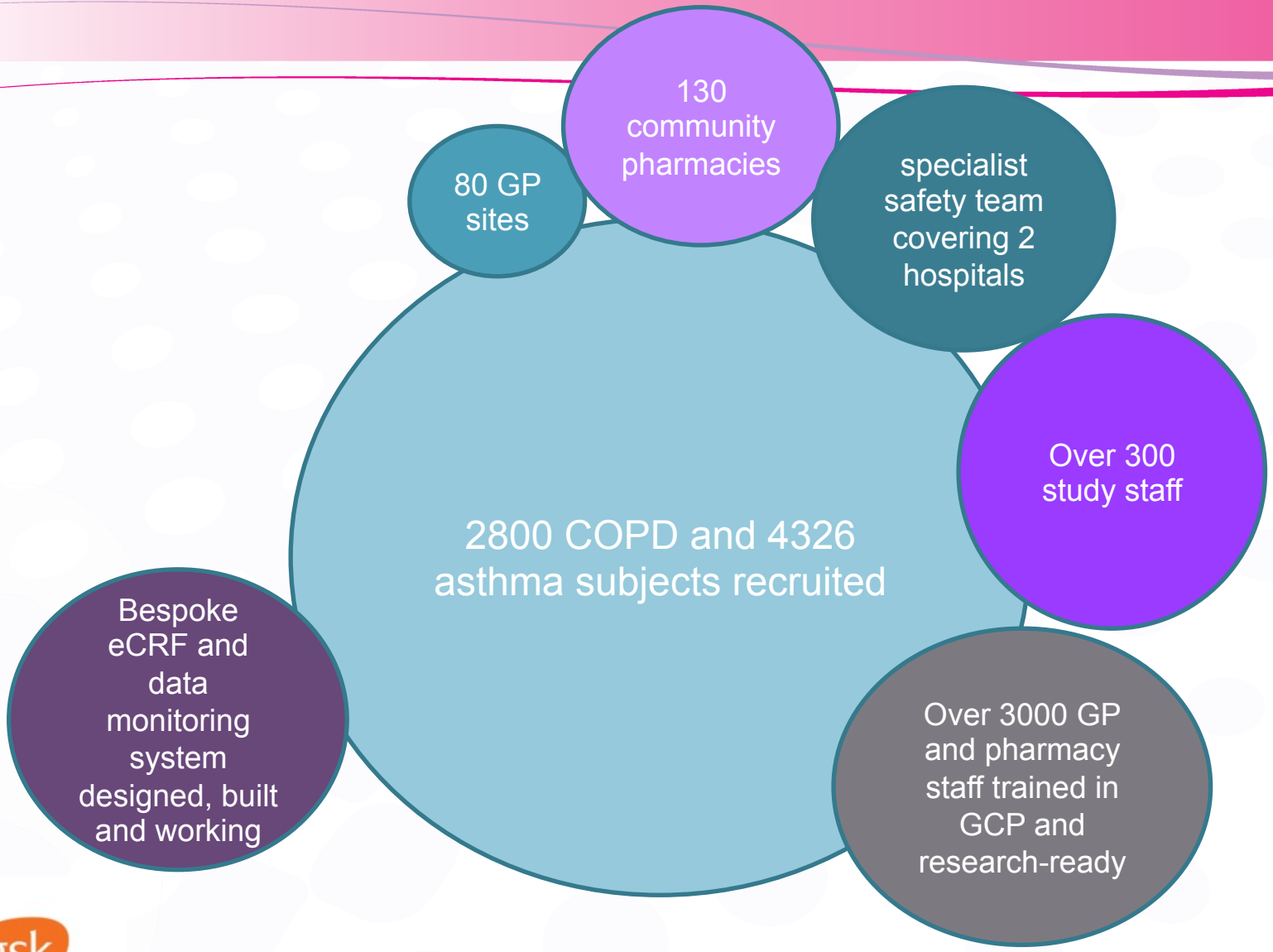
- How to recruit patients?
  - "all comers"
  - broad inclusion criteria
  - pragmatic diagnostic criteria
  - few exclusions
- How to ensure "normal" care of patients during the study?
  - minimal study procedures
  - normal prescribing and dispensing practices
- How to monitor patients without carrying out frequent reviews?
  - minimize "Hawthorne" effect
  - ensure patient safety
  - ensure robust collection of end points

- Recruit patients through primary care
- No additional review
- No change to "care as usual"
- Study drug accessed through "high street" community pharmacy network
- Integrated electronic patient record (EMR) with real-time access ensures that data is complete wherever and whenever patient accesses healthcare

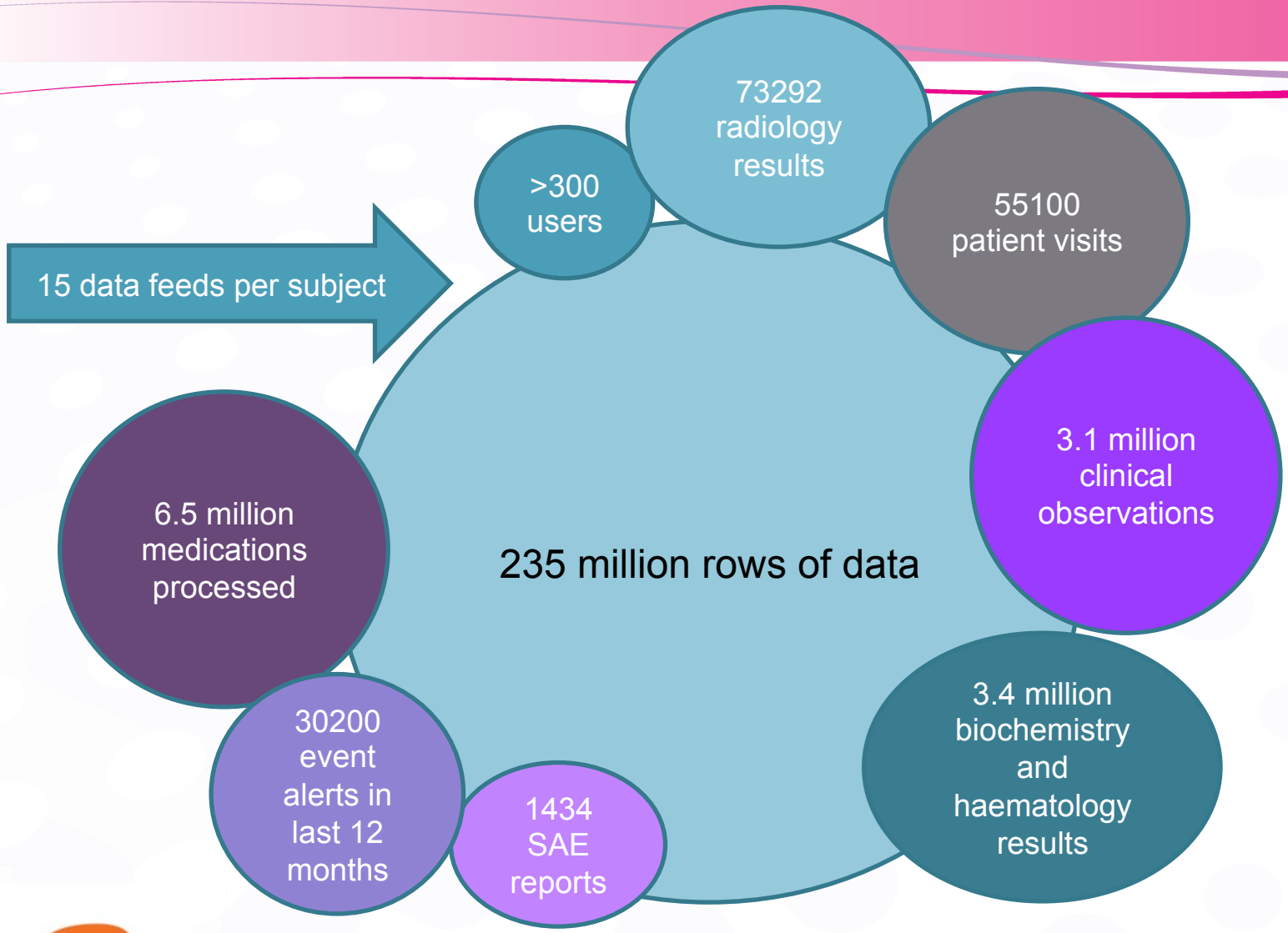
How the data is gathered



Scale of the Project



Electronic Clinical Monitoring



How will this look in the future?

- Partnerships – NHS, Industry, Patients
- Culture of research – physicians and patients
- Operationally complex – opportunity to simplify
- Data Flows – how do we scale? Quality & Reliability
- Improving the care of patients through evidence