Medicines & Healthcare products Regulatory Agency

Use of synthetic data in clinical trials: A stakeholder's journey to overcome limitations of standard clinical trials

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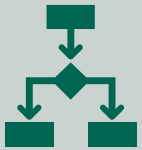
Outline

The problem to be addressed

Aims and objectives



Journey

Introduction

The pharmaceutical industry is urging regulators to explore new approaches to overcome the limitations of standard clinical trials, particularly for rare or life-threatening diseases and experimental treatments with high safety concerns.

- Generalisability and real-world application
- Lack of diversity
- Cost and time
- Outcome and risk

This project builds upon our previous RPF-funded regulatory science work by exploring how properly utilised synthetic data can help fill evidence gaps, potentially accelerating or facilitating the approval and licensing of new treatments.

What are the problems to be addressed?

The main motivation of this programme is to address the limitations of standard clinical trial methods when dealing with

- rare and/or life-threatening diseases
- experimental treatments with high safety concerns and unproven benefits

This proposal was developed in response to feedback from clinical trial sponsors on these challenges and their request for regulatory guidance on the use of synthetic data in clinical trial.

Aim and objectives

Aim

- Creation of a regulatory sandbox where two industry partners will work with regulatory experts on how synthetic data can be incorporated into their clinical trial protocols to address current clinical trial challenges.

Objectives

- To enable synthetic data to support clinical trials involving human participants for the first time
- To progress the safe and effective development of the medicines supported by the sandbox
- To develop the first regulatory guidance on the use of synthetic data in clinical trials

What are the expected outcomes?

1. This project will enable the first use of synthetic data to support clinical trials involving human participants and inform regulatory guidance on its appropriate use and quality requirements.

2. This innovation could accelerate the development of safe and effective treatments, particularly for rare or life-threatening diseases, by reducing reliance on control arms that may withhold potentially life-saving treatments.

3. It could improve patient outcomes, shorten development timelines, reduce trial costs, and position the UK as a global leader in synthetic data-enabled clinical trial innovation.

Sandbox

A **sandbox** is an isolated, safe environment used to test, experiment, or play without causing real-world damage

- Regulatory Flexibility
- State Oversight
- Time-Limited
- Evidence-Based Policymaking

Synthetic data Regulatory Sandbox

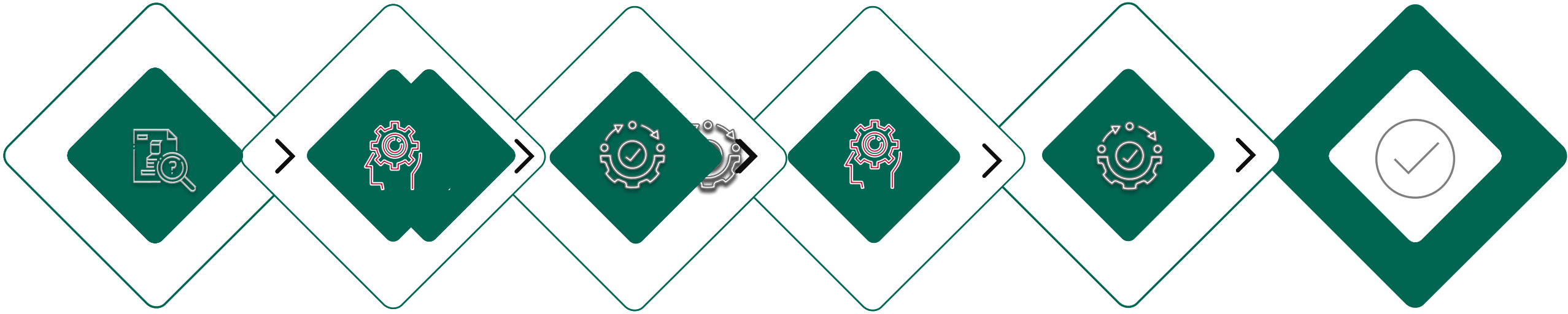
Industry partners working with MHRA regulatory experts on how synthetic data can be incorporated into clinical trial protocols to address current clinical trial challenges.

Two industry partners are participating.

Case study 1 focuses on how synthetic data could be used to simulate treatment efficacy and safety profiles in underrepresented age groups, while allowing for extrapolation from adult data.

Case study 2 focuses on how synthetic patient cohorts can be used as external control arms (ECAs) to enhance the capture of treatment responses across the full spectrum of patient variability.

Our journey involved more than 150 people



20 November 2025: Kick-off workshop

Background to the program and initial presentations

December 2025: Stakeholder follow-up meetings

Individual company meetings to discuss next steps

27 January 2026: Second workshop

Detailed stakeholder updates with real data

February 2026: Stakeholder follow-up meetings

Individual company meetings for more detailed discussion

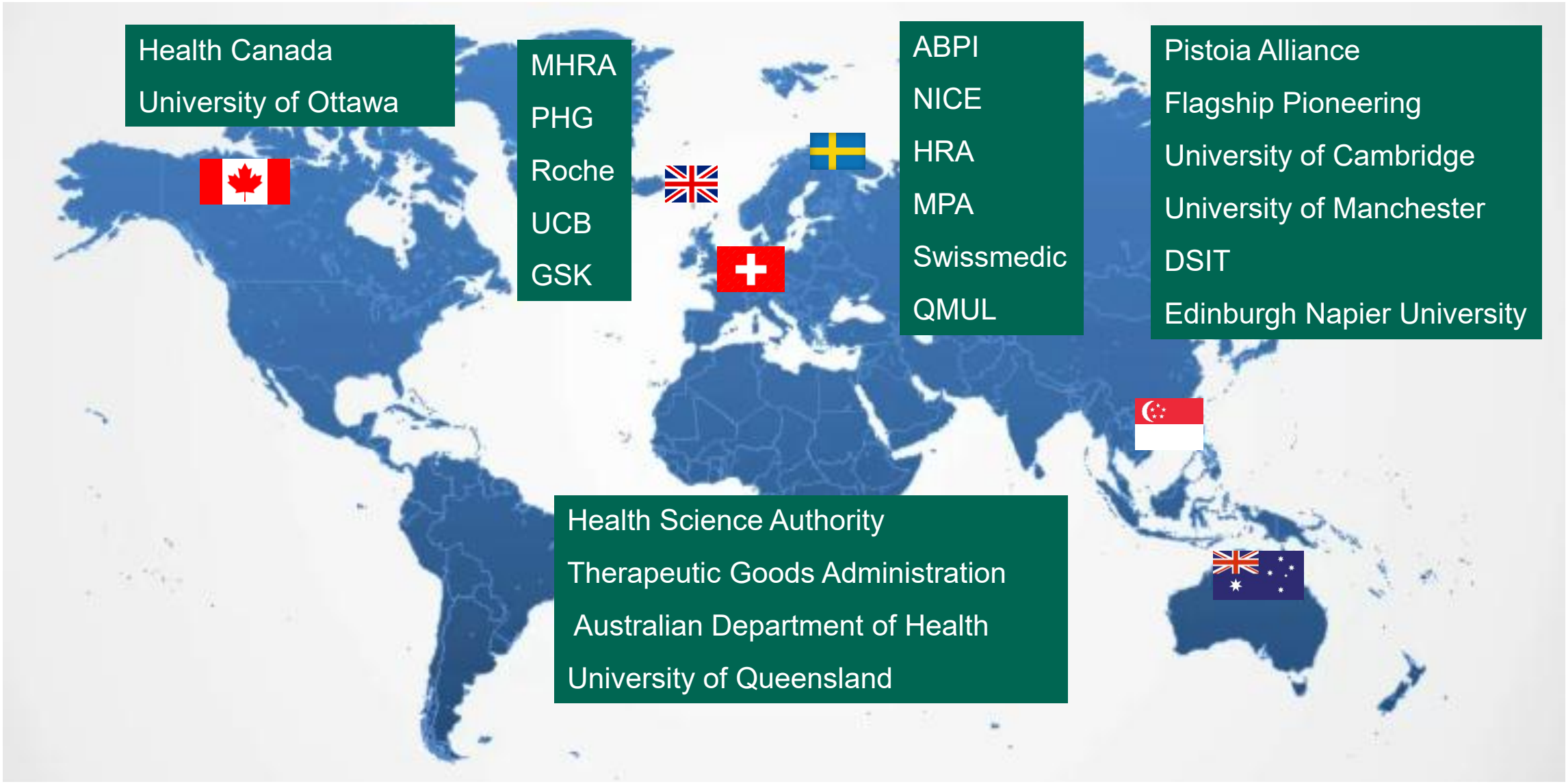
2 March 2026: Third workshop

Additional stakeholders. MHRA initial considerations.

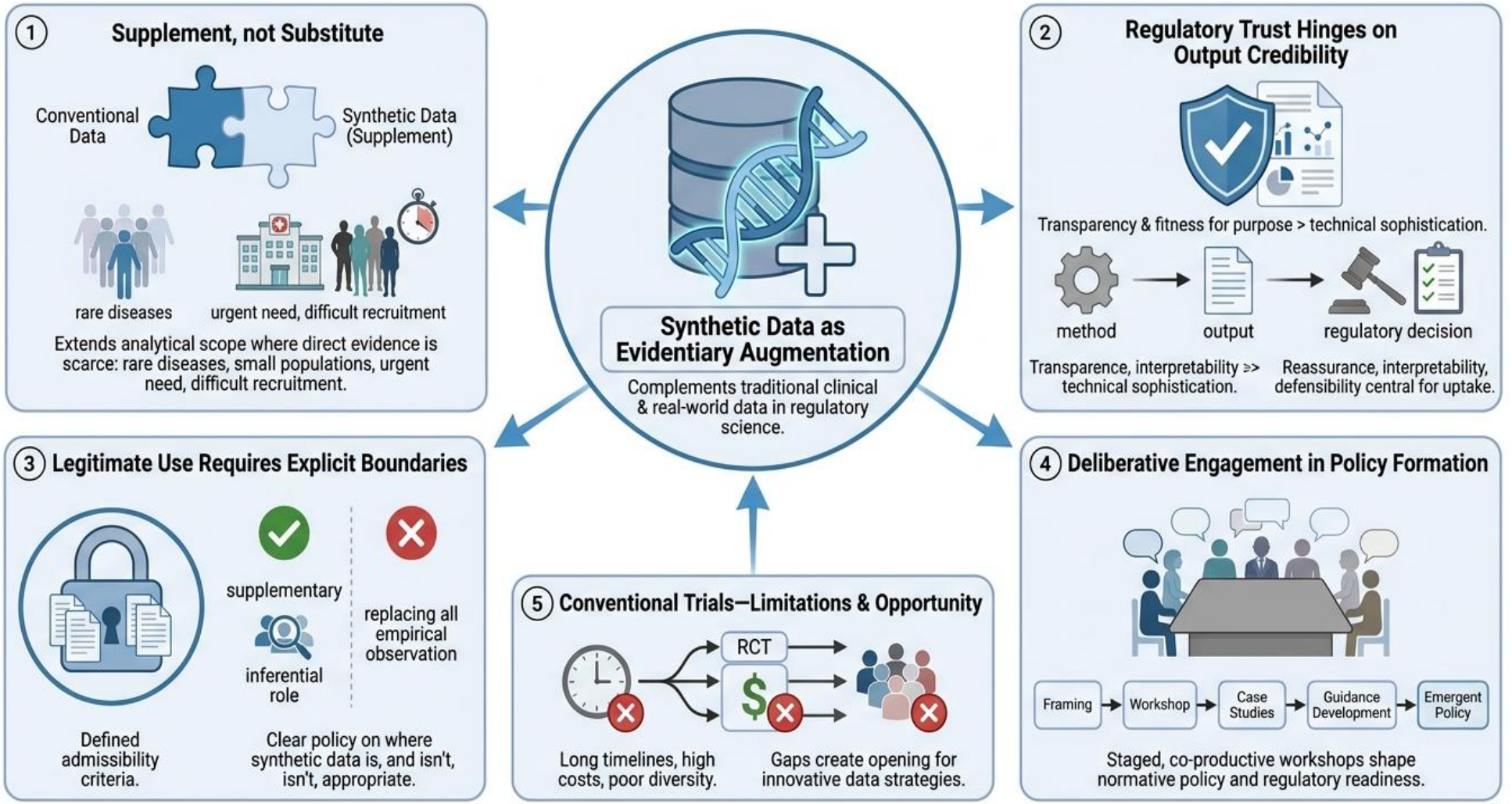
2 and 3 July 2026: Fourth workshop

Case studies presentation. Discussion considerations

Attendee list for Day 1 international workshop



Role of synthetic data as an evidentiary augmentation strategy in regulatory and policy



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Thank you

Any questions?

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