Medicines & Healthcare products Regulatory Agency

MEB-CCMO Science Day

Learnings on Rare Disease: A pilot program for rare therapies and individualised medicines in the United Kingdom

Julian Beach

20 May 2026

We have delivered strong results in 2025/26

We are
hitting all our targets

Staff morale
has risen

External sentiment
has improved

We are
financially stable

We are
leading in key areas of regulatory science

We have built
closer national and international partnerships

Fast and reliable performance: what we've done

Clinical trial and clinical investigation applications assessed on time

Financial year (FY) 24/25 KPI performance

March 2026 KPI performance

National medicines licence applications determined on time

Recognition of international medicines licences determined on time

Scientific advice requests — written advice delivered on time

Decisions on manufacturing and distribution authorisations made on time

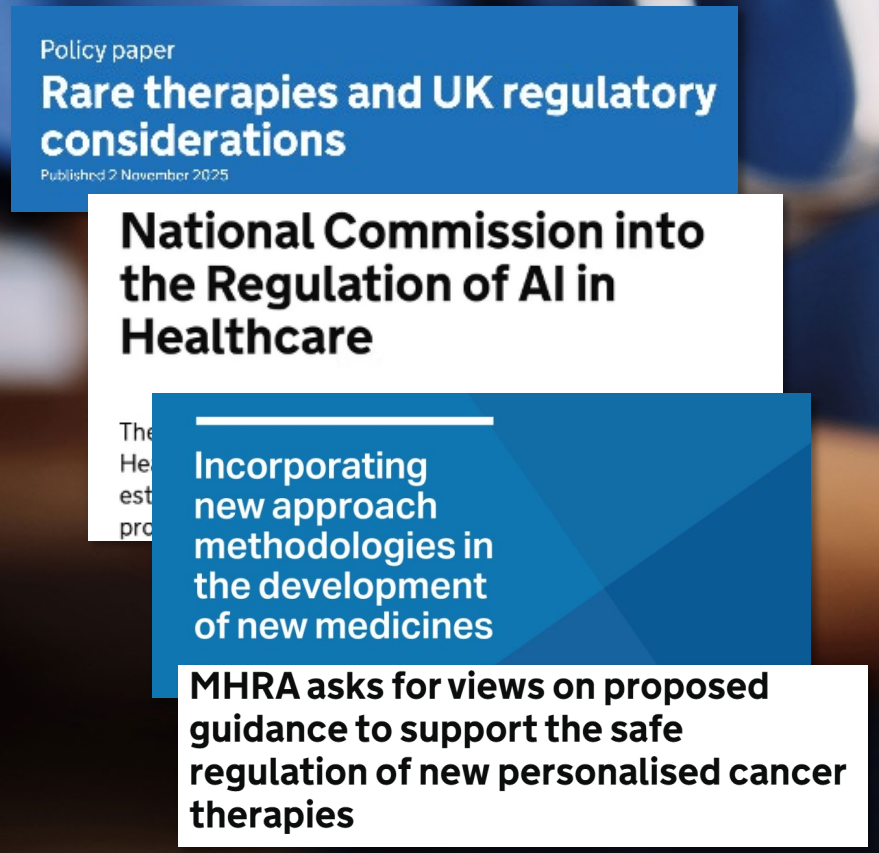
8/8

Key Performance Indicators (KPIs) met in 2026

"The MHRA is now delivering consistently good approval times while introducing further reforms to add speed and flexibility to the process."

Lord James O'Shaughnessy on MHRA CT approvals

Specialisation and innovation: what's next



Rare therapies

AI innovation and leadership

Novel Approach Methodologies (NAMS)

Personalised pathways

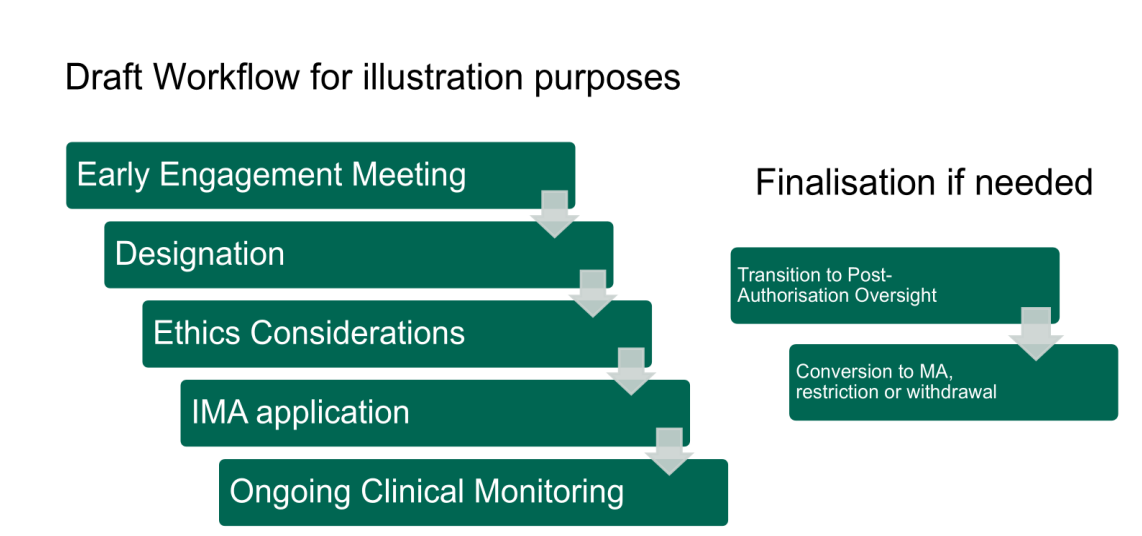
90day first assessment for NAS

I was heartened to read the recent MHRA paper which lays out a new vision for rare disease therapies. These reforms will speed up the time from discovery to delivery, and these changes directly give families like mine hope for a brighter future.

Chris Kessler, parent of Charlie

Regulatory Thinking and next steps

Rare Diseases: A lifecycle framework that accelerates access, preserves rigour, and aligns the system



Personalised Immunotherapies: Draft guidance published with consultation completed. Updates on the product design to include GMP control of AI process steps, with final publication expected in Q2 2026

Underpinning concepts being developed: technology-agnostic to enable proportionate regulation using risk-appropriate evidence, prior and predictive knowledge, with platform inclusion

The Rare Therapies Launch Pad (RTLP)

Rare Therapies Launch Pad

The RTLP aims to establish a scalable and sustainable new pathway for individualised medicines, focusing on putting in place an innovative regulatory and reimbursement system to allow for rapid access to anyone who could benefit.

RTLP Mission and Collaborators

Rare Therapies Launch Pad

- The RTLP is a multi-stakeholder consortium working to support the sustainable and equitable build of the necessary infrastructure and processes to deliver individualised genetic therapies at scale:
  - Allow patients with no/few existing options to gain rapid access to cutting-edge science
  - Condition, age and treatment modality agnostic
  - Facilitate the switch from research innovation to patient treatment with iterative learning
- Goals and ambitions outlined in *Nature Medicine* in 2025
- Contributing to the England Rare Diseases Action Plan 2025-27

Genomics England

abpi

University of Oxford

Oxford-Harrington Rare Disease Centre

Mila's Miracle Foundation

Great Ormond Street Hospital Charity

OHE Office of Health Economics

University of Birmingham

The Yu Lab

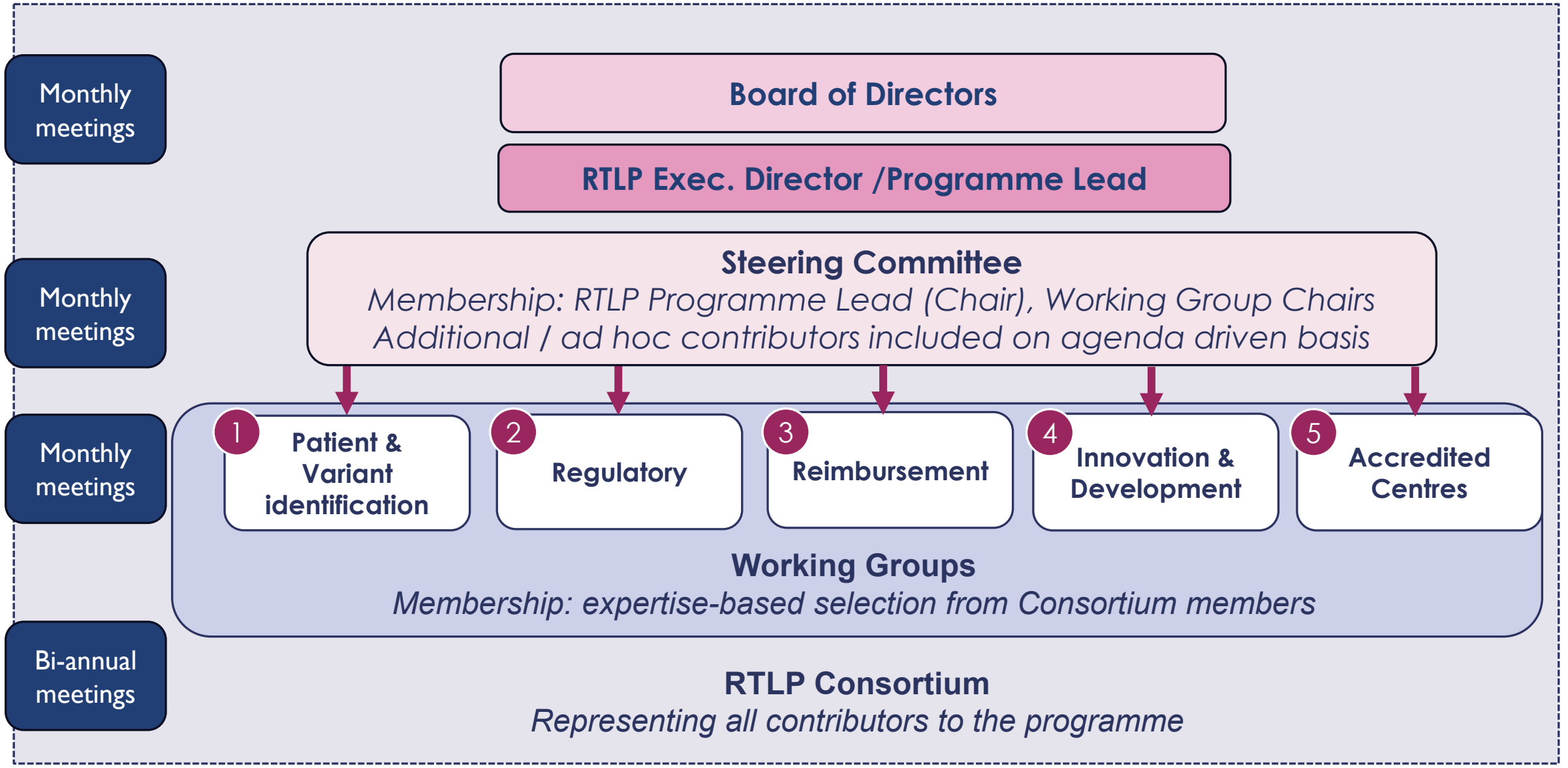
Genetic Alliance UK

UCL

Other collaborators: MHRA, DHSC, NHSE, RDR UK

RTLP Governance Structure

Rare Therapies Launch Pad



RTLP Objectives

Rare Therapies Launch Pad

- **Establish a new end to end pathway:** Define resources, infrastructure, and data - applicable across conditions and therapeutic modalities – that can enable the shift from research to treatment with iterative learning.
- **Map Stakeholder Responsibilities:** Clarify roles for clinicians, families, industry and manufacturers researchers, the NHS, regulators, and payers, with appropriate risk and accountability sharing.
- **Identify Policy Gaps:** Highlight where policy, regulatory, or legislative updates are needed, especially around oversight, access, and implementation.
- **Position the UK as a global leader in the development and access to genetic medicines:** partner with innovators, manufacturers and clinical delivery teams to create the tools and ecosystem for world-leading, timely and sustainable reimbursable access to individualised medicines, grounded in robust quality, ethics and compliance.

RTLP consensus position statements

Rare Therapies Launch Pad

- Existing Regulatory frameworks do not fully achieve the mandate to protect public health, as access to life-saving technologies is being impeded for patients with unique or extremely rare mutations

- To be an enabler of innovation, we believe that a significant change in regulatory process could be the driving force for change, and allow patients access to the treatments they desperately need

- There is an urgency to develop a new pathway due to the high unmet medical need and the mismatch between what is technically feasibly with 21st century science and what is currently available

- RTLP considers that the regulatory framework must be responsive and conducive to the evolving landscape of personalised medicine, facilitating innovation and safe patient access

We have the tools to build on

Rare Therapies Launch Pad

We can borrow from existing precedents, e.g. stem cell transplant settings, where clinical centres of excellence allow for individualised, adaptable risk assessment and consent processes:

- Adjust risk-tolerance to individual priorities in the context of life-limiting or fatal rare diseases

Designated (accredited) Individualised Medicines Centres treating patients within pre-defined health system parameters:

- Can be informed by prior evidence and experience
- Enable opportunities for data extrapolation across platform technologies and basket / umbrella protocols
- Allow for proportionate regulatory and access flexibilities

A system of accreditation agreed collectively by the health system could provide accountable, reimbursable process within a mandatory central framework for data collection and iterative learning.

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Thank you