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Rare Disease Research UK.

UK Platform for Nucleic Acid Therapies - UPNAT

UPNAT Node news | Summer 2025

England Rare Disease Action Plan 2025


Department of Health & Social Care

Policy paper

England Rare Diseases Action Plan 2025: main report

Published 28 February 2025

The UPNAT Node has been included in the Government’s 2025 Action Plan, recognising the government’s significant investment in individualised therapies.

The Action Plan highlights UPNAT’s role in the operational framework for individualised therapies in the NHS, and identifies key priorities such as:

- building a national network for knowledge and expertise sharing between leading nucleic acid therapy (NAT) preclinical and clinical development centres
- facilitating systematic linking of patients who have unique genetic defects to NAT expertise
- streamlining the regulatory approvals process by supporting discussions between regulators and researchers
- accelerating the clinical translation of rare disease specific NATs

To find out more about the UPNAT Node’s 2025 commitments, read the England Rare Disease Action Plan: www.gov.uk/government/publications/england-rare-diseases-action-plan-2025/england-rare-diseases-action-plan-2025-main-report

Poster Award at the Genomics England Research Summit 2025

Congratulations to Dr Ella Whittle for receiving the Best Poster Award at the Genomics England Research Summit 2025. Ella, who is a Research Fellow for the UPNAT Node, presented a poster titled “Developing Target Selection Guidelines for Nucleic Acid Therapies".

This prestigious award was announced by Professor Matt Brown, CEO of Genomics England. Over 45 posters were displayed, covering a wide range of critical topics such as rare disease

The associated publication, which builds on the N1C Consensus Guide for assessing eligibility of pathogenic DNA variants for antisense oligonucleotide treatments, is expected later this year. The UPNAT Guidelines are tailored for implementation in the UK, with the addition of assessments across disease, patient, and functional model.

More information about the Genomic England Research Summit 2025 programme and posters can be found here:
https://genomicsresearchsummit.co.uk/genomicsengland2025posters

More information about the UPNAT Node poster can be found here:
https://rd-research.org.uk/uncategorized/upnat-wins-best-poster-award-at-genomics-england-research-summit/

UPNAT Symposium
– 21 November 2025 (London)

We are grateful to UPNAT Node members and partners for their participation at the upcoming UPNAT Node Symposium.

We look forward to welcoming an excellent group of speakers, which includes:

- **Professor Matt Brown** (Genomics England, CEO) will deliver a keynote presentation on translational genomics and rare diseases at Genomics England.
- **Dr Kath Bainbridge** (DHSC), who helped shape the2025 Action Plan, will review the rare disease landscape.
- **Dr Rachel Smith** (Paraxel) will share her experience working in every development phase of rare disease and cell and gene therapy clinical trial with Parexel’s rare disease clients.
- **Dr Marlen Lauffer** (Dutch Center for RNA Therapeutics) brings her expertise on developing individualized genetic therapies for patients with rare neurological disorders and assessment for patient cases and determining the eligibility towards a genetic therapy.
- **Dr Alban Ziegler** (University Hospital of Toulouse) will discuss his work on characterizing the natural history of several rare genetic disorders, in particular antisense oligonucleotide therapy in an individual with KIF1A-associated neurological disorders.
- The **Centre for Process Innovation** (CPI) will introduce their UK-based Good Manufacturing Practice site of RNA Centre of Excellence.

More details about the programme will be released closer to the time, but we hope you will save the date for the **UPNAT Node Symposium on 21 November 2025**.

Research UK.

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