

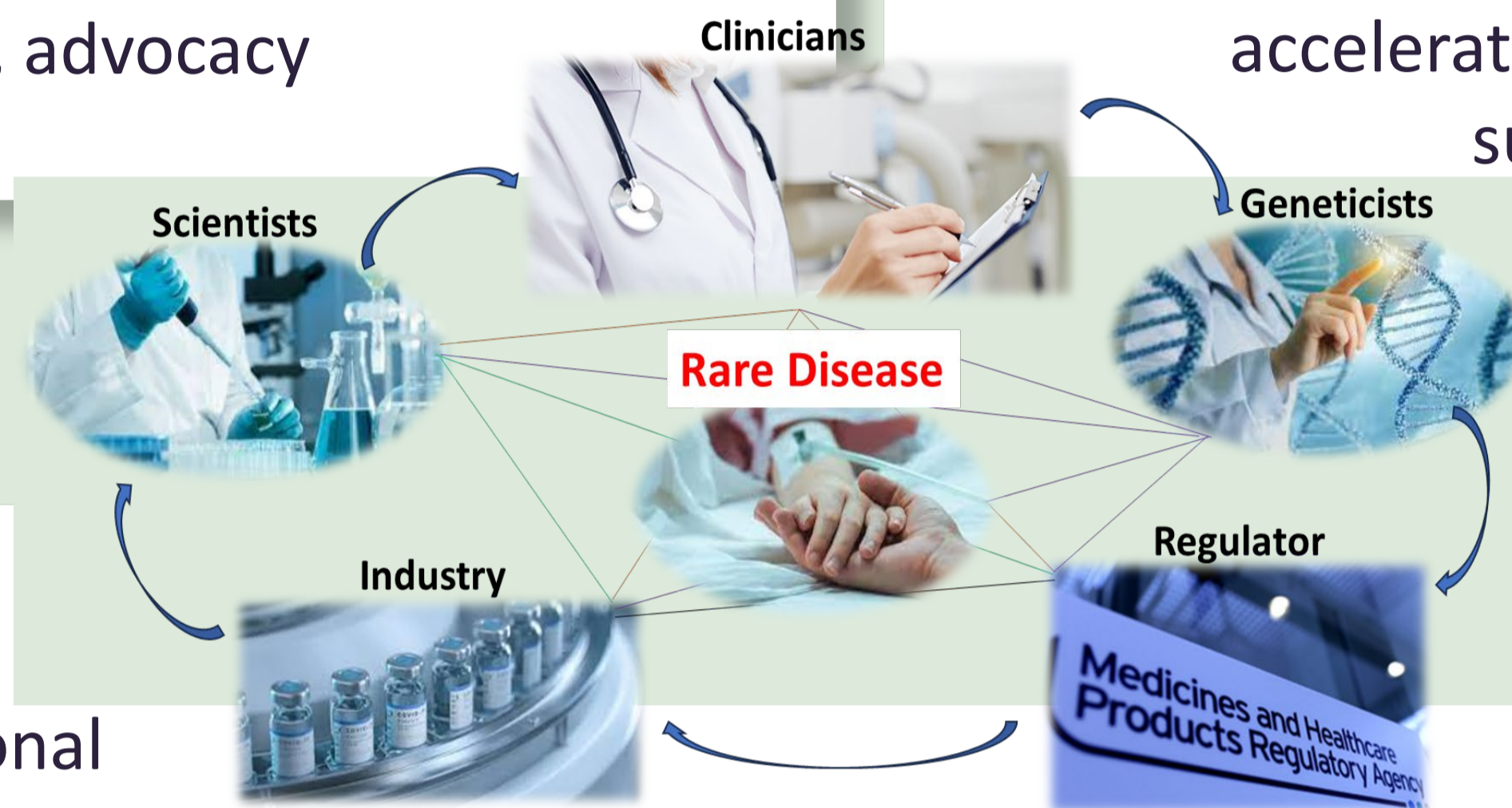
UPNAT is a **UK Platform of Nucleic Acid Therapy (NAT)** for Rare Diseases (RD), funded by the **Medical Research Council (MRC)** and the **National Institute for Health and Care Research (NIHR)**, as part of the **Rare Disease Research UK Platform** launched in July 2023 (<https://rd-research.org.uk/>).

Bring together scientists, geneticists and clinicians to generate experimental guidelines and promote knowledge exchange on NAT development for RD, and promote communications, public engagement and raise awareness for experimental NAT development among RD patients, families, charities, advocacy groups and clinical centres.

Work closely with regulatory authorities to establish and publish clear guidance on RD NAT preclinical development, clinical trial design principles and regulatory approval applications, to streamline the process and accelerate the clinical translation of such therapies in an efficient and safe manner.

Objectives

Link and seek further collaboration with academic groups, industry and international consortia to explore NAT treatments in diverse disease areas, with particular emphasis on rare disease.



Enabling Projects

Target Selection and NAT Pre-clinical Development

NAT scale-up manufacturing and Toxicity studies for RD

NAT clinical trial and regulatory approval

Outcomes

- ✓ NAT rare disease target selection guidelines
- ✓ National rare disease NAT development database
- ✓ Proof-of-concept pre-clinical NAT data

- ✓ A pilot scheme to test NATA's manufacturing partner ecosystem
- ✓ A whitepaper detailing standardised toxicology NAT assessment
- ✓ Pipeline to link clinicians and scientist to real-world NAT manufacturing systems

- ✓ White papers on preclinical requirements for regulatory approval and clinical trial design principles on RD NATs
- ✓ Expert recommendations on guidelines to endpoints in clinical NAT development
- ✓ Forum to provide feedback to regulators and ethic committees
- ✓ Curated registry of adverse events in NAT investigator-initiated studies

Our vision

Provide a roadmap for rapid bench-to-bedside translation of NATs for Rare Disease

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