

**Rare Disease  
Research UK.**



**UK Platform for Nucleic  
Acid Therapies - UPNAT**



# UPNAT Inaugural Symposium

27 June 2024, 10am-5pm

UCL Institute of Child Health

Kennedy Lecture Theatre

30 Guildford Street, London, WC1N 1EH

JOINTLY FUNDED BY



Medical  
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**NIHR**

National Institute for  
Health and Care Research

Registration is now open:

[click here \(Eventbrite\)](#)

to reserve a space

[UPNAT@ucl.ac.uk](mailto:UPNAT@ucl.ac.uk)

The agenda will include sessions on:

## Real-world experience from patients & clinicians

- Piotr Kosla (parent, and Simons Searchlight Community Advisory Committee member)
- Francesco Muntoni (Chair of Paediatric Neurology, UCL)

## Target selection

- Stephan Sanders (Professor of Neurogenetics, Oxford)
- Ana Lisa Taylor Tavares (Clinical Lead for rare disease research, Genomics England)

## Clinical trials and regulatory path

- Sarah Tabrizi (Professor of Clinical Neurology, UCL)
- Daniel O'Connor (Director of Regulatory and Early Access Policy, Association of the British Pharmaceutical Industry)
- Willeke van Roon-Mom (Professor of Human Genetics, Leiden University)

**+Panel discussion**, chaired by Paul Gissen (Clinical Professor of Paediatric Metabolic Medicine, UCL GOSH)