From Model to Therapy

hDMT/RARE-NL Preclinical Network

When	Wednesday 11 March 2026, 9:00-13:30
Where	Location: LUMC (Burumazaal, Onderwijsgebouw)
For whom	Preclinical researchers and stakeholders interested in therapy development for rare diseases.

Programme

9:00 - 9:30 | Arrival with coffee and tea

9:30 - 9:45 | Introduction
9:45 - 10:45 | Examples therapy development

Ex vivo gene therapy - Pim Pijnappel (Lenticure)
AON development - Rob Collin (Astherna)

10:45 - 11:00 | Break

11:00 - 11:30 | Regulatory expectations
11:30 - 12:30 | Interactive workshop session, lessons learned

12:30 - 13:30 | Lunch

Register here:

A RARE-NL meeting on n-of-few AONs will take place in the afternoon of the same day. For more information see this page

www.rare-nl.nl

RARE-NL

Leids Universitair Medisch Centrum

Antisense oligonucleotides for n-of-few: on the way to patients

Wanneer? Wednesday 11 March 2026, 13.30 – 16.30
Waar? Burumazaal, Onderwijsgebouw LUMC, Leiden

This RARE-NL meeting on 11 March at LUMC focuses on the development and implementation of antisense oligonucleotides for n-of-few and n-of-1 indications. The meeting brings together researchers, clinicians and experts in access pathways to explore how AONs can be translated into clinical practice.

13.30 – 14.45

Opening	Sibren van den Berg (RARE-NL) & Dirk Lefeber (Radboudumc | RARE-NL)
ASO-landscape	Willeke van Roon-Mom (LUMC | DCRT)
Block 1: Production & infrastructure	Martin Pool (LUMC) & Tobias Pöhlmann (XNApharma)

14.45 – 15.15 | Break

15.15 – 16:30 | Part 2

Block 2: Clinical implementation	Wouter van Rheenen (UMCU) & Bart van de Warrenburg (Radboudumc)
Block 3: Regulatory & Reimbursement	Marjon Pasmooij (CBG), Joop van Gerven (METC Amsterdam UMC) & t.b.d.
Plenary reflection and outlook	

16.30 – 17.30 | Drinks & networking

Register here:

The hDMT/RARE-NL *preclinical network meeting* will take place in the morning of the same day. For more information see this page.