



# LEUKODYSTROPHIES: NOVEL PERSPECTIVES ON TREATMENTS AND DISEASE MONITORING COURSE:

SAVE THE DATE



**25-27 SEPTEMBER 2025**  
**PARIS, FRANCE**

## SCIENTIFIC

## Organising Committee:



### **FANNY MOCHEL**

Professor of Medical Genetics and  
Neurometabolism.  
ICM & Sorbonne University, Paris, France



### **NICOLE WOLF**

Professor Therapy of Leukodystrophy  
Amsterdam UMCAmsterdam, the  
Netherlands



### **CAROLINE SEVIN**

Clinical Expert Paediatric Neurology,  
Reference Center for Leukodystrophies.  
Kremlin-Bicêtre hospital, Bicêtre, France

## FOR PAEDIATRIC & ADULT

Neurologists,  
Neuroradiologists,  
Paediatricians,  
Geneticists, Internists  
and Neuroscientists

**For more information  
and registration:**



**rrd-foundation.org**

Contact: [ckellquist@rrd-foundation.org](mailto:ckellquist@rrd-foundation.org)

## COURSE OVERVIEW AND LEARNING OBJECTIVES:

- Introduction to **leukodystrophies**: definition, classification, mri patterns, physiopathology, main entities.
- New **molecular** and **imaging tools** to **diagnose** and **monitor disease** progression in leukodystrophies.
- Update on existing and emerging treatments in leukodystrophies (**cell therapy, gene therapy, small molecules**).
- Ethical challenges: burden of long-term **MRI monitoring**, impact of **newborn screening**, burden of **therapeutic trials** for children.



Applicants  
are strongly  
recommended  
to apply with a  
case before  
**27 JULY 2025**

Leukodystrophies, inherited white matter disorders, require differentiation from acquired forms. This course explores their complex pathophysiology, including various cell and tissue involvements. Emphasis will be placed on novel biological and imaging markers for monitoring disease and treatment response. The increasing therapeutic options, such as cell and gene therapy, and the ethical implications of new-born screening programs will also be discussed.

