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the European Union**

General Assembly Meeting

21-22-23 October 2026

Agenda

This event will be recorded. The public session recording will be available on ERDERA communication channels. The closed ERDERA consortium meeting sessions recording will only be available to ERDERA consortium partners.

Programme at a glance

October 21st 2026 Morning

Governing Board (GB) and Operating Group (OG) ONLY

Venue: Coimbra, Portugal – Vila Galé – Romeu Room

Time (WEST)	Title	Speakers	Room (Link to be sent separately)
08:30 - 09:00	<i>Registration: Governing Board (GB) and Operating Group (OG) members</i>		
GB & OG closed session: strategic discussions for ERDERA			
09:00 - 10:30	Interactive engagement methodology that looks at how the GB and the OG view ERDERA at the half-way mark	GB & OG members	Romeu
10:30 - 11:00	<i>Coffee and network break - GB Group Photo & portraits</i>		
11:00 - 11:45	Policy role of ERDERA	GB & OG members	Romeu
11:45 - 12:30	ERDERA financial management & payment distribution	GB & OG members	

October 21st 2026 Afternoon

Venue: Coimbra, Portugal – Vila Galé – Romeu Room

Time (WEST)	Title	Speakers	Room, Teams Link
12:30 - 13:30	Registration and lunch		
Official Opening (public session, Session 1)			
13:30 - 14:15	WELCOME	João Ramalho-Santos , Vice-Rector for Research, Coimbra University. Daria Julkowska, PhD. ERDERA Coordinator, INSERM Tomasz Dylag , European Commission, DG Research and Innovation Dusan Sandor , European Commission, DG Research and Innovation HaDEA	Romeu, Public Session Link

14:15 – 15:00	<i>Investigator-Initiated Path to Genetically-Targeted Therapies in Prion Disease: A Patient-Scientist Case Study</i>	Pr. Sonia Vallabh, PhD. Director, Prion Therapeutic Science, Broad Institute, Boston MA, USA <i>(remote)</i>	
15:00 - 15:45	<i>Regulatory Preparedness for the Next Generation of Rare Disease Therapies: Innovation, Evidence and Patient-Centered Development</i>	Julian Beach , Medicines and Healthcare products Regulatory Agency, UK <i>(Remote)</i>	
15:45 - 16:30	<i>European framework for N-of-1 treatments Updates from ERDERA WP12</i>	Annemieke Aartsma-Rus, PhD. Professor of translational genetics. Department of Human Genetics. Leiden, Netherlands	
16:30 -17:00	<i>Coffee and network break - Group Photo & portraits</i>		
Closed ERDERA Consortium meeting			
17:00 - 17:15	Session 2: European Commission introduction	Tomasz Dylag , European Commission, DG Research and Innovation Dusan Sandor , European Commission, DG Research and Innovation, HaDEA Ioannis Lismanis , European Commission, DG Research and Innovation, HaDEA	Romeu, Link for sessions
17:15 -17:45	Session 3: Behind the Scenes of ERDERA: a Year of Coordination	Coordination Unit	
17:45 - 18:30	Session 4: Communication Updates	Jordi Vaqué , TEAMIT Freya Sentmarti , TEAMIT Gisela Paró , TEAMIT	

October 21st 2026 Evening

Venue: Quinta das Lagrimas, Coimbra, Portugal

Closed ERDERA Consortium meeting	
20:00 – 22:30 (WEST)	<i>Networking Dinner: Informal Networking and Discussions</i>

October 22nd 2026

Venue: Coimbra, Portugal – Vila Galé.

Time (WEST)	Title	Speakers	Room, teams Link
08:30 - 09:00	Registration		
Plenary Sessions			
09:00 - 09:45	Session 5: Sustainability	Malwina Mejer, CE	Romeu, Link for sessions
09:45 - 10:45	Session 6: Everything you want to know about DSH services	DSH WP Leaders	
10:45 - 11:00	Coffee and network break		
11:00 - 12:00	Session 7: Sustainability of Education & Research – Shaping the Next Generation of Rare Diseases Researchers	Uğur Özbek, IBG Wanda Lattanzi, UCSC Prabha Rana, UCSC Levent Altıntaş, ACU Kaan Mert Güven, ACU	Romeu, Link for sessions
12:00 - 12:35	Session 8: Inclusion by Design: Mechanisms, Assets and Mutual Benefits of Underrepresented Countries	Anabela Isidro, AICIB Maja Stojiljkovic, IMGGE, Naima Fdil, CAU	
12:35 - 12:55	Session 9a: Exploring ERDERA Funded Projects through the PURE Dashboard	Max Dumoulin, Elsevier	
12:55 - 13:15	Session 9b: OIP Discovery: Supporting ERDERA Activities with AI	Robin Sarfati, TEKKARE	
13:15 - 14:00	Lunch Break		
Breakout Sessions			
14:00 - 15:30	Session 10: alignment session in GAM about the collaboration between NCATS RD CRNs and ERDERA’s CRN & DSH	WP13-WP16 Representatives from ERDERA’s CRN, NCATS and the U.S. RDCRNs	Quixote + Paquita (Room 2), Link for session
14:00 - 15:30	Session 11: JTCs in Phase 2: Opportunities and Challenges (Closed Session for Funders)	WP3 Board of Funders Coordination Unit	Gisele + Copelia (Room 3), link to be sent separately

15:30 - 16:00	Coffee and network break		
MAB + ExCom closed Session (Session 12, Romeu Room)			
16:00 - 17:00	New EU pharma reform package	MAB & ExCom	Romeu, link to be sent separately
17:00 - 18:00	How to engage with WHO	MAB & ExCom	

October 23rd 2026

Venue: Coimbra, Portugal – Vila Galé.

Time (WEST)	Title	Speakers	Room, Teams Link
08:30 - 08:45	Registration		
Breakout Sessions			
08:45 - 10:15	Session 13: From Therapeutic Opportunity to Translational Readiness: Aligning Platforms, Evidence and Methodologies	WP11, WP12, WP19, WP21	Quixote + Paquita (Room 2), Link for session
08:45 - 10:15	Session 14: Federated analysis in ERDERA: CRN use cases and DSH support	WP6 – WP15	Gisele + Copelia (Room 3), Link for session
10:15 – 10:30	Coffee and network break		
Breakout Sessions			
10:30 - 12:00	Session 15: DSH internal meeting: data services and the VP	WP13, 14, 15, 16 leaders	Quixote + Paquita (Room 2), Link for session
10:30 - 12:00	Session 16: n-of-1 / of-few trials.	WP12 – WP18 – WP19	Gisele + Copelia (Room 3) Link for session
Plenary Session (Romeu Room)			
12:00 - 12:45	Session 17: Closing Session	Coordination Unit	Romeu, Link for session
12:45	Lunch bags		

Programme at a glance

October 21st 09:00 – 12:30 WEST

GB & OG closed session: *strategic discussions for ERDERA*

Chair/co-chair

Hugh Dawkins, GB Chair and Iveta Hermanovska, GB vice-chair

Objectives

Establish a shared understanding of:

- Where ERDERA stands today and how its evolving strategic environment may influence its future direction
- The specific roles of different ERDERA bodies
- ERDERA's strategic positioning for its next phase: policy goals, priorities and strategy in the global context
- The role the GB should play in shaping future strategic choices and forthcoming SRIA revision
- ERDERA financial management and payment distribution

Description

The first part of the session will be divided into two blocks:

- 1) An overview of where ERDERA started and how it's been consolidated in two years, with an initial presentation of the different governance structures and of the strategic context in which ERDERA is evolving
- 2) An interactive workshop with GB and OG to define governance roles and their evolution as ERDERA progresses strategically. The workshop will be based on Tricker's Framework, which is described in the pre-reading for this session.

The second part of the session focused on ERDERA's policy role will be complementary to the first part. In addition to defining what is meant by ERDERA playing a role in policy discussions, the new WP1 subtask dedicated to policy strategy will be presented. Ultimately, if the topic has not yet been addressed during the previous part, using Tricker's as a foundation, a strategic discussion will take place to define the role the GB should take in shaping ERDERA's policy strategy and positioning.

The third part of the session will provide the Governing Board with an overview of ERDERA's financial management processes, including the current cash-flow situation, the roles of the internal actors involved in the payment workflow, and opportunities to improve payment efficiency and predictability across the consortium.

Given the size and complexity of the consortium, ensuring adequate cash-flow visibility for beneficiaries remains a challenge. The discussion will focus on feedback received from beneficiaries regarding treasury planning, as well as recurring issues related to reporting deviations, data corrections, and payment timelines.

The Governing Board will be invited to discuss concerns raised regarding recent payment delays and to consider potential measures to improve coordination, communication, and planning throughout the payment process. The session will also address the role of Smartsheet and the Financial Dashboard in supporting financial monitoring, reporting, and transparency.

October 21st 17:00 – 17:15 WEST

Session 2: European Commission introduction

Chair/co-chair

Dusan Sandor, European Commission, DG Research and Innovation, HaDEA

Objectives

Description

October 21st 17:15 – 17:45 WEST

Session 3: Behind the Scenes of ERDERA: a Year of Coordination

Chair/co-chair

ERDERA Coordination Unit, INSERM&TEAMIT

Objectives

Provide a retrospective overview of the first year of ERDERA implementation, highlighting the key coordination efforts, achievements, lessons-learned, and improvements introduced to support the consortium and ensure the successful delivery of project activities.

Description

This session will reflect on the first year of ERDERA from the perspective of the Coordination Team. It will present the main accomplishments, challenges addressed, and operational improvements implemented over the past. The session will offer insights into how coordination has evolved and how these experiences will inform the next phase of the project.

October 21st 17:45 – 18:30 WEST

Session 4: Communication Updates

Chair/co-chair

Jordi Vaqué, TEAMIT - Freya Sentmarti, TEAMIT - Gisela Paró, TEAMIT

Objectives

This presentation hopes to help align all ERDERA partners with WP2's C&D priorities as the project enters a critical 3rd year with a high volume of deliverables, milestones, and expected results. It also seeks to reinforce the shared responsibility of communication, encouraging partners to report activities early, acknowledge ERDERA support, and actively act as ambassadors for the initiative. The session also aims to demonstrate how effective planning, coordination, and engagement with WP2 can increase the visibility, reach, and impact of ERDERA's work.)

Description

The presentation provides an overview of ERDERA's current communications ecosystem, Year 2 performance indicators, and the strategic pathway towards greater impact and recognition. Through practical examples it highlights successful communication approaches, identifies common gaps in reporting and collaboration, and explores how partners can better contribute to dissemination efforts. The session concludes with a forward look at 2027 communication priorities, planned campaigns, and opportunities to strengthen ERDERA's position as a leading initiative in rare disease research.

October 22nd 09:00 – 09:45 WEST

Session 5: Sustainability

Chair/co-chair

Malwina Mejer – Copenhagen Economics

Objectives

Provide an update on ERDERA sustainability activities and discuss the next steps towards ensuring the long-term sustainability of ERDERA's outputs and ecosystem.

Description

Sustainability is essential to maximise ERDERA's long-term impact and maintain a coherent rare disease research ecosystem. Copenhagen Economics has developed the ERDERA Phasing-Out Strategy Document and conducted an analysis of sustainability questionnaires across ERDERA Workstreams. This session will present the main results of this work and outline the next steps for strengthening ERDERA's sustainability.

October 22nd 09:45 – 10:45 WEST

Session 6: Everything you want to know about DSH services

Chair/co-chair:

DSH WS Leaders: Ana Rath – INSERM Orphanet, Ronald Cornet – Amsterdam UMC (AUMC), Dimitrios Athanasiou – World Duchenne Organization

Objectives

Showcase how the DSH supports researchers in practice, moving beyond the service catalogue to real-world applications.

Description

Key message: What can the DSH do for you? Practical examples demonstrating how ERDERA researchers, registries, biobanks, and CRNs can benefit from DSH services.

- Overview of the DSH **catalogue of services**.
- Presentation of the service request workflow, ticketing system, and open office support model.
- Demonstration of concrete use cases such as onboarding, biobank data integration, FAIR Data Points, CRN requests, and data readiness for federated analysis.
- Focus on data quality, interoperability, and data readiness services.
- Illustration of how WP13-14-15-16 services are being applied through **tangible examples** and ongoing collaborations.

Program of the Session:

- Services presented by WP leaders: Overview of the DSH service catalogue and the support services available across the different work packages.
- General workflow (Alberto Camara Ballesteros, Universidad Politécnica de Madrid): Presentation of the DSH service delivery process, including service requests, ticketing, and Open Office support,
- Followed by practical use cases demonstrating DSH services in action.

October 22nd 11:00 – 12:00 WEST

Session 7: Sustainability of Education & Research – Shaping the Next Generation of Rare Diseases Researchers

Chair/co-chair

Uğur Özbek, IBG TR - Wanda Lattanzi, UCSC (online)

Objectives

How do we train the next generation of rare disease researchers? Task 20.4 set out to answer this by developing a European University Diploma in rare disease research. Through needs analysis, educational design and consortium consultation, the idea evolved into two complementary components: the ERDERA Label, recognising existing postgraduate programmes that provide strong researcher training and meaningful opportunities to undertake rare disease research; and ERDERA Training Modules, providing focused rare disease learning that complements formal academic programmes and supports the ERDERA diploma pathway. Both are designed to grow with the contributions of ERDERA partners.

Description

The European Labelling Programme team (WP 20.4) will explain why the approach moved from a stand-alone diploma towards labelling existing programmes and shared modules, and what the ERDERA Label, Training Modules and pilot will look like in practice. We will also look at where and how ERDERA partners can get involved in the future. This is more than a presentation of outputs: it is an invitation to help shape the system and build shared ownership of it beyond the current project period.

October 22nd 12:00 – 12:35 WEST

Session 8: Inclusion by Design: Mechanisms, Assets and Mutual Benefits of Underrepresented Countries

Chair/co-chair

Anabela Isidro, AICIB - Maja Stojiljkovic, IMGGE - Naima Fdil, CAU

Objectives

Discuss how ERDERA Workstreams can better identify, engage and leverage Underrepresented Country assets and capacities to strengthen collaboration, participation and impact across the Partnership.

Description

This session will explore how ERDERA can better leverage the expertise, capacities, and perspectives available within Underrepresented Countries, while creating conditions for their meaningful participation across the Partnership. The presentation will provide brief updates on WP24 activities, achievements, and upcoming plans. Particular attention will be given to the assets and capacities identified through Task 24.2. The discussion will focus on practical approaches to strengthening participation, fostering collaboration, and ensuring that the expertise available across Underrepresented Countries is effectively integrated and used throughout the Partnership.

October 22nd 12:35 – 12:55 WEST

Session 9a: Exploring ERDERA Funded Projects through the PURE Dashboard

Chair/co-chair

Max Dumoulin, Elsevier

Objectives

- Demonstrate how PURE can help capture, track and showcase the impact of JTC-funded projects across projects.
- Collect feedback from the partners on future developments.

Description

This session will present the ERDERA PURE platform and demonstrate how data from JTC-funded projects can be transformed into a searchable and interconnected knowledge resource. Using the one project as a use case, the presentation will showcase links between projects, researchers, publications and funders, while highlighting opportunities for impact monitoring, visibility of research outcomes and future collaboration

October 22nd 12:55 – 13:15 WEST

Session 9b: OIP discovery: Supporting ERDERA activities with AI

Chair/co-chair

Robin Sarfati, TEKKARE

Objectives

- Present OIP Discovery and its key capabilities relevant to ERDERA stakeholders.
- Showcase OIP Discovery AI approaches for leveraging both public and confidential data.
- Collect ideas and identify potential use cases to support ERDERA work packages and future activities.

Description

This session will introduce OIP Discovery and illustrate how AI-powered tools and data intelligence can support ERDERA activities. Through concrete examples, participants will explore opportunities to accelerate research, identify expertise and collaborations, analyze scientific and clinical landscapes, and securely leverage data to support decision-making and innovation across ERDERA.

October 22nd 14:00 – 15:30 WEST

Session 10: Alignment session in GAM about the collaboration between RDCRNs and ERDERA's CRN & DSH

Chair/co-chair

- DSH WS Leaders (Ana Rath – INSERM Orphanet, Ronald Cornet – Amsterdam UMC (AUMC), Dimitrios Athanasiou – World Duchenne Organization)
- Franz Schaefer, UKHD
- Joni Rutther, NIH NCATS RDCRNs

Objectives

- Compare ERDERA and NCATS RDCRN approaches to rare disease research, outcome research, and evidence generation.
- Exchange experiences on data harmonization, regulatory and methodological approaches, and the development of research-support tools.
- Identify areas of alignment and complementary strengths across ERDERA, the RDCRNs, and the DMCC.
- Explore concrete opportunities for collaboration, knowledge exchange, and future joint activities. Define priority topics and next steps for focused follow-up between the ERDERA and RDCRN communities.
- between NCATS RDCRNs, ERDERA's CRN and the DSH.

Description

This interactive alignment session will bring together NCATS, U.S. NIH Rare Disease Clinical Research Networks, and ERDERA representatives. Following an overview of the respective network models and activities, participants will discuss potential matches between RDCRN consortia, ERDERA CRNs, and DSH's use cases to identify practical opportunities to renew and strengthen transatlantic collaboration."

October 22nd 14:00 – 15:30 WEST

Session 11: The strategic direction of JTCs in Phase 2 and how to maximize funders engagement and ERDERA synergies

Chair/co-chair

Ralph Schuster, DLR – Pauline Nauroy, FFRD - Florence Guillot, ANR

Objectives

CLOSED SESSION

Description

CLOSED SESSION

October 22nd 16:00 – 18:00 WEST

Session 12: MAB + ExCom closed Session

Chair/co-chair

Eleni Grigorakaki, Dariusz Adamczewski, Lina Basel

Objectives

Part I: New EU Reform Package:

- Examine implications of the Pharmaceutical Reform Package for rare disease research and development, identifying key challenges and opportunities for ERDERA.

Part II: ERDERA's engagement with WHO

- Determine together how ERDERA can establish a structured engagement process with WHO to contribute to the Global Action Plan on Rare Diseases (GAPRD)

Description

The first part of this session will provide an overview of the key elements of the EU Pharmaceutical Reform Package, with a particular focus on their implications across the rare disease research and drug development pathway. The discussion will explore how the reform may affect research, development, regulatory incentives, and market access, and will invite participants to reflect on ERDERA's

potential role in supporting implementation and addressing emerging challenges and opportunities.

The second part of the session will introduce the evolving WHO rare disease landscape and ongoing global initiatives, creating a shared understanding of the current context and how ERDERA members are currently participating in different WHO initiatives. Through interactive discussions, participants will identify ERDERA's unique strengths and potential contributions to WHO objectives, prioritize areas for engagement, and formulate recommendations for future collaboration and strategic action.

October 23rd 8:45 – 10:15 WEST

Session 13: From Therapeutic Opportunity to Translational Readiness: Aligning Platforms, Evidence and Methodologies

Chair/co-chair

Luís Pereira de Almeida, UC - Laetitia Gaspar, UC

Objectives

Year 3 marks an important transition from platform and methodological development towards **integration, prioritisation and translational readiness**. This breakout session will bring together WP11, WP12, WP19 and WP21 to:

- **Establish a common understanding of the Year 3 translational pathway**, connecting disease prioritisation, technology platforms, methodological support and individualized therapeutic approaches.
- **Define the interfaces between the four WPs**, including the information, evidence and methodological support that each WP needs from, and can provide to, the others.

Agree on concrete coordination priorities for M25-M36, identifying key dependencies, responsibilities and next steps towards the M36 outputs and subsequent Phase 2 activities

Description

With the start of Year 3, **WP11, WP12, WP19 and WP21** will increasingly work at the interface of their activities. Rather than focusing on individual WP progress, the session will explore how these WPs should interact to support ATMP development, individualized therapies, technology platform deployment, and evidence generation in rare disease settings.

Short contributions from each WP will clarify:

- What is already available;
- What will be delivered during Year 3;
- What support or inputs are needed from the other WPs.

These contributions will serve as the basis for an interactive discussion to identify information flows, methodological requirements, dependencies, and gaps

across the four WPs, with the objective of defining effective collaboration mechanisms for the remainder of the project.

Expected outcomes

- A shared map of the main **WP11-WP12-WP19-WP21 interfaces**;
- An initial definition of **who needs what from whom, for which purpose and by when**;
- A set of **priority actions and coordination mechanisms for M25-M36**.

October 23rd 8:45 – 10:15 WEST

Session 14: Federated analysis in ERDERA: CRN use cases and DSH support

Chair/co-chair

Sergi Beltran Agullo, CNAG - Morris Swertz, UMCG

Objectives

- Present the Data Services Hub (DSH) portfolio relevant to federated analysis and the Group 3 use cases.
- Illustrate each use case through practical examples.
- Clarify how the DSH can support these use cases.
- Identify the requirements, potential solutions, and immediate next steps needed to initiate collaboration.

Description

This session will explore how federated analysis can support ERDERA CRN research use cases, particularly in genomics and multiomics. The DSH portfolio and the Group 3 use cases will be presented using practical examples. Participants will discuss methodological needs, available DSH support, potential solutions, and the concrete actions required to move the use cases forward.

October 23rd 10:30 – 12:00 WEST

Session 15: DSH internal meeting: data services and the VP

Chair/co-chair

Ana Rath, INSERM Orphanet - Ronald Cornet, AUMC

Objectives

This internal DSH meeting aims to review progress, define concrete next steps, assign responsibilities, and align contributors around actionable deliverables and timelines.

Description

A strategic planning session focused on advancing key DSH priorities, including patient data readiness, registry integration, interoperability, EHDS alignment, and federated analysis.

DSH members will review progress, identify gaps and bottlenecks, agree on concrete actions, assign responsibilities, and establish clear next steps to drive implementation **across DSH work packages**.

October 23rd 10:30 – 12:00 WEST

Session 16: n-of-1 / n-of-few trials.

Chair/co-chair

Matthis Synofzik, UT – Viviana Giannuzzi, FGB - Geert Molenberghs, UHASSELT

Objectives

Develop the current conceptual trial synopsis (step 1) into a sufficiently concrete & data-driven trial synopsis (step 2). This shall establish a sufficiently robust basis for early, but still meaningful & promising discussions with regulators, so that the approach is not turned down too early or moved in wrong directions.:

- **Set 1: Collaboration with WP19:** Data-driven effect sizes, Severity / endophenotypes, Per trial arm and across trial arms, Analysis approaches.
- **Set 2: Collaboration with regulatory advisors + WP18:** Platform identity, Transfer of prior knowledge & tiered safety model, Expandable master protocol, Adding new ASO arms.

Description

This session will present an innovative umbrella trial concept designed to evaluate individualized therapies through an expandable master protocol. Using Ataxia-Telangiectasia as a use case, the discussion will explore how collaboration between WP12, WP18 and WP19 can help transform this concept into a data-driven and regulatory-ready clinical trial framework for rare diseases

October 23rd 12:00 – 12:45 WEST

Session 17: Closing Session

Chair/co-chair

ERDERA Coordination Unit

Objectives

Synthesize the key outcomes of the General Assembly discussions and identify the main priorities, opportunities, and actions emerging from the breakout sessions held throughout the event.

Description

This closing session will provide a consolidated overview of the General Assembly. Chairs and speakers from the different plenary and breakout sessions will present the main discussion points, conclusions, recommendations, and proposed next steps from their respective sessions. The session will highlight the key take-home messages from the event and provide participants with a shared understanding of the priorities and actions that will guide ERDERA's activities in the coming months.