ERDERA European Rare Diseases Research Alliance

ERDERA POLICY BRIEF

MAKING ALL DATA COUNT FOR RARE DISEASE RESEARCH AND HEALTH OUTCOMES

From policy recommendations to implementation

Executive Summary

Europe already has many of the standards and policy frameworks needed to improve the use of rare disease data. The remaining challenge is implementation: making high-quality, interoperable and reusable data a routine output of research and clinical practice.

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This brief identifies four priorities:

1 **Require good data practice.**
Funders should embed regulatory readiness, data quality and interoperability into funding conditions and project expectations, including use of common standards and demonstrable interoperability. *(Recommendations 1.1, 2.3, 2.4, 3.1, 4.4)*

2 **Fund the enablers of responsible data use.**
Regulatory support, automation, workforce, EHDS readiness, data curation and interoperability need to be treated as legitimate and eligible research costs rather than unfunded expectations. *(Recommendations 1.2, 2.1, 2.2, 2.3, 3.1, 4.3, 4.5)*

3 **Enable implementation.**
Regulators, and their relevant authorities, should provide clear legal and regulatory guidance for cross-border data use. Research organisations (including infrastructures) should develop, share and support practical frameworks for cross-border data use. *(Recommendations 1.2, 1.3, 1.4, 2.1, 2.2, 2.3, 2.4, 2.5, 4.2)*

4 **Reward implementation, stewardship and partnership.**
Data quality, interoperability and stewardship should be properly considered in project assessment. Patient-led data registries and patient organizations should be recognized as substantive collaborative partners in the research and participating families should receive meaningful feedback. *(Recommendations 3.1, 4.1, 4.2, 4.3, 4.4, 4.5)*

In other words, **funders must require, fund and reward good practice; regulators and competent authorities must provide clearer pathways; research infrastructures and organisations must enable implementation; and consortia and registries must put these expectations into practice.**

> **Europe does not need another generation of recommendations. It needs coordinated implementation of what is already known to work.**

Purpose of this document

This policy brief translates existing recommendations on rare disease data[1] into practical actions for implementation. It identifies **what needs to change, who needs to act and which existing policy, funding and infrastructure levers can be used** to make the generation of high-quality, interoperable and reusable data a standard output of rare disease research and clinical practice.

The aim is not to develop new principles or standards, but to help move from **recommendations to coordinated implementation**, so that every data point generated through the participation of patients and families can contribute to better research, regulatory, HTA and healthcare decisions.

The problem we want to address

Rare disease data are inherently scarce: patient populations are small, dispersed across countries and health systems, and every data point generated through the participation of patients and families is therefore particularly valuable for research and to inform regulatory decision-making. Yet too much of these data cannot be effectively reused because implementation remains fragmented and despite the existing recommendations approaches to data collection, access, sharing, and reuse remain fragmented across countries and health systems.

Experience, as well as the conclusions of the May 2026 Policy Think Tank workshop on data, show that research projects and registries face inconsistent interpretation and application of regulatory and legal requirements, uneven data quality and interoperability, limited access to specialist expertise and infrastructure, and insufficient resources for data stewardship. Healthcare professionals face significant data-capture burdens, while patient contributions and the work required to curate, share and reuse data are not consistently recognised or rewarded.

In rare diseases, data are the rarest commodity, and we cannot afford to waste any of the data that patients have painstakingly helped to generate. The challenge is therefore not to create another set of standards, but to align requirements, funding, enabling infrastructure and incentives so that high-quality data can be responsibly reused and translated into research, regulatory, HTA and healthcare impact.

1 Examples of existing recommendations include: European Patients Forum (2025) EPF's analysis of the European Health Data Space Regulation; Friends of Europe (2021) Building a path for rare diseases in the European Health Data Space; Kole, A., Hedley V., et al. (2021); Recommendations from the Rare 2030 Foresight Study: The future of rare diseases starts today; Sitra and TEHDAS Consortium Partners (2023); Advancing data sharing to improve health for all in Europe: Main findings of joint action Towards the European Health Data Space 2021-2023; Tarride, J., Okoh, A., Aryal, K. et al. Scoping review of the recommendations and guidance for improving the quality of rare disease registries. Orphanet J Rare Dis 19, 187 (2024).

Why Europe remains stuck

Europe remains stuck because existing standards, principles and recommendations are not implemented consistently; implementation is insufficiently supported and funded, and there are few effective incentives for compliance.

Technical and semantic standards (including codification systems), FAIR principles, Common Data Elements and regulatory guidance largely exist[1]. Current barriers are the cumulative product of fragmented implementation, legal uncertainty, insufficient regulatory readiness of data sets, uneven technical maturity and funding models that do not reward high-quality reusable data[2]. Patient organisations remain outside the governance of the registries that their communities populate and lack data access on the same terms as researchers, while registries themselves depend on short-term project funding that threatens long-term data availability[3]. The EHDS, current and future EU research funding programmes, the Biotech Act and EU-funded initiatives such as ERDERA and JARDIN provide an opportunity to address these barriers. The urgency of implementing these measures is further underscored by the EU's increasing focus on AI-enabled healthcare through effective data use. This was illustrated by European Commission President von der Leyen's State of the Union address on 16 September 2026 highlighting health as one of the priority sectors for accelerating the uptake of AI.

To address implementation gaps, Europe needs three complementary types of intervention:

ENABLERS
Build capability, trust and coordination.

IMPLEMENTATION ACTIONS
improve operational practice.

POLICY REFORMS
Embed long-term change through legislation, governance and funding.

No single intervention will be sufficient. Progress depends on combining all three.

1 dos Santos Vieira, B., Bernabé, C.H., Zhang, S. *et al.* Towards FAIRification of sensitive and fragmented rare disease patient data: challenges and solutions in European reference network registries. Orphanet J Rare Dis 17, 436 (2022); EMA/152628/2024. Real-world evidence provided by EMA: Support for regulatory decision-making; Kölker S, Gleich F, Mütze U and Opladen T (2022) Rare Disease Registries Are Key to Evidence-Based Personalized Medicine: Highlighting the European Experience. Front. Endocrinol. 13:832063. doi: 10.3389/fendo.2022.832063; Vohora A., Maiella S., Lucano C., Kirch K., Thomas C., Weber S., Rath A. Rare disease nomenclature and coding: challenges, systems, and policies in the global context. Archives of Medical Research, Volume 57, Issue 8 (2026).

2 Ines de Oliveira Coelho Henriques, Valentina Sand, Daniela Vicentini Albring et al. Rare Disease Data Management across Europe: Insights from the JARDIN Survey on Data Sharing and Interoperability, 01 December 2025, PREPRINT (Version 1) available at Research Square.

3 EURORDIS (2011). Patients' priorities and needs for rare disease research 2014-2020; Hageman, I.C., van Rooij, I.A., de Blaauw, I. et al. A systematic overview of rare disease patient registries: challenges in design, quality management, and maintenance. Orphanet J Rare Dis 18, 106 (2023).

Theme 1 Improve regulatory readiness and maximise regulatory impact

Strategic objective: ensure that publicly funded research consistently generates data fit for regulatory, HTA and healthcare decision-making.

1.1 Require regulatory readiness in funding calls [Policy reform]



Challenge: Current funding frameworks do not consistently incentivise or require regulatory planning, early regulatory engagement, data-quality standards or future evidence reuse, resulting in missed opportunities to maximise the long-term value and regulatory utility of research data and patient contribution (i.e. data are not fit for submission to product approval or other regulated processes such as marketing authorisation or health technology assessment).



Recommendation: Mandate regulatory readiness in funding calls with minimum data quality standards and mechanisms to monitor the implementation of such standards. Attach a regulatory-readiness checklist to call texts as a planning tool, requiring data-generating projects to consider potential future regulatory use and identify the regulatory expertise and standards relevant to their project. Evaluate explicitly the regulatory expertise available and resourced in each project and, for projects generating clinical or outcome data, whether patient-relevant outcomes have been defined with patient organisations.



Responsible actors: research funders (incl. European Commission, national agencies, foundations, ERDERA).



Implementation lever: Leverage Rules for Participation and Work Programmes for the 10th Framework Programme for Research and Innovation (FP10/Horizon Europe), and ERDERA annual call conditions and evaluation criteria to integrate regulatory readiness, data-quality expectations, future evidence reuse, and recognised standards for inter-operability, terminology and disease classification, including ORPHAcodes and ICD-11, into research funding and evaluation frameworks. Minimum data-quality requirements should build on, and not duplicate, existing frameworks, including the EHDS Data Quality and Utility Label and the EMA Data Quality Framework, as applicable.



Future state: Regulatory readiness becomes a standard expectation of publicly funded research, with projects routinely incorporating regulatory strategies, data quality planning and future reuse considerations from proposal stage. Publicly funded research consistently generates interoperable, reusable and regulatory-ready data-sets because minimum expectations for data quality and stewardship are embedded in funding programmes. **[policy]**

1.2 Provide early regulatory support [Enabler]



Challenge: Researchers often lack structured (early) regulatory guidance during proposal development and project design, limiting their ability to align evidence-generation plans with future regulatory and HTA requirements across a fragmented methodological landscape.



Recommendation: Provide or fund access to regulatory support and advice from the early stages of projects (e.g., at the proposal stage) to ensure that regulatory strategies are incorporated in project design and implementation from the start (engagement with regulatory agencies, and/or regulatory affairs consultants, regulatory service platforms where applicable). Create or fund regulatory support platforms with regulatory affairs specialists who can provide support during the entire project lifecycle. Project proposals must demonstrate how such support or advice will be used throughout the project.



Responsible actors: Research Funders (incl. ERDERA), EMA, national HTA Bodies.



Implementation lever: Build on ERDERA regulatory support services, EMA Scientific Advice pathways and European and national HTA advice mechanisms to provide structured early guidance that aligns research design with future regulatory, and HTA evidence needs.



Future state: Researchers have timely and predictable access to regulatory expertise throughout the project lifecycle, resulting in fewer missed opportunities to generate evidence suitable for multiple downstream uses. Study designs reflect patient priorities and outcomes from the outset, ensuring that valuable patient contributions translate into evidence that is relevant for regulatory, HTA and healthcare decision-making. **[practice change]**.

1.3 Develop regulatory case studies [Enabler]



Challenge: Rare disease registry data remain under-utilised in research, healthcare and decision-making. Limited visibility of successful examples contributes to uncertainty about how registry and other real-world data can be used effectively, including for regulatory and HTA purposes, and can weaken confidence in investing in regulatory-ready data.



Recommendation: Develop and publish case studies and best practices demonstrating how registry data and other real-world data sources have been successfully used to support research, healthcare, regulatory and HTA decision-making, including examples from registries established and governed by patient organisations.



Responsible actors: ERDERA, EMA.



Implementation lever: ERDRI, ERDERA Data Hub / Virtual Platform and established real-world evidence initiatives, including EMA RWE Framework (DARWIN EU), as well as internationally recognised guidance as a reference such as the CIOMS Working Group report on Real-world data and real-world evidence in regulatory decision making.



Future state: A growing body of documented examples demonstrates how high-quality registry data support regulatory, HTA and healthcare decisions, increasing confidence among researchers, regulators and funders in investing in regulatory-ready data. **[practice change]**.

1.4 Harmonise GDPR guidance [Enabler]



Challenge: Researchers, clinicians, patient organisations and companies continue to face inconsistent legal and governance requirements due to divergent interpretations of data-protection rules across institutions and countries. The current data and registry regulatory environment is ambiguous and subject to inconsistent interpretations that confound implementation and collaborative aspirations, and lead to fragmentation of data collection and use, and barriers to cross-border data access, sharing and reuse.



Recommendation: Provide clear, harmonised outcomes-based guidance for institutional Data Protection Officers (DPOs) on the application of data-protection law (GDPR) to rare disease research to reduce divergent institutional interpretations that fragment requirements within multi-site consortia and delay data sharing. Guidance must also address patient organisations acting as data controllers for the registries they establish, curate and maintain.



Responsible actors: EDPB supported by other competent authorities at European Commission and Member States level.



Implementation lever: Use EHDS Implementing Acts, EDPB guidance (see e.g. Guidelines on processing of personal data for scientific research purposes and Commission guidance for Health Data Access Bodies.



Future state: Institutions implement a more consistent and standardized interpretation of GDPR and EHDS requirements, reducing delays in multicentre research, enabling predictable cross-border data sharing and driving innovation and new knowledge translation. **[system change]**.

Theme 2 Build technical and legal/institutional support infrastructure

Strategic objective: remove operational barriers to interoperability while strengthening governance and public trust.

2.1 Reduce healthcare professional burden [Implementation action]



Challenge: Healthcare professionals face high burden related to data entry. The absence of harmonised data platforms and tools, standards, training and integration with routine care reduces clinician engagement and undermines data quality and completeness.



Recommendation: National competent authorities should fund the workforce, tools and automation needed for efficient data capture at the point of care and integration with routine clinical workflows, aligned with relevant EHDS interoperability requirements. In parallel, they should support – alongside hospital managers, HCPs, researchers and EHR vendors – the co-design and adoption of EHR systems that align with clinical workflows and incorporate rare disease ontologies.



Responsible actors: National competent authorities responsible for health, digitalisation, data economy; EHR vendors, and hospital managers.



Implementation lever: EHDS implementation, national digital health programmes, Horizon Europe digital health calls, public procurement requirements.



Future state: Clinical data collection becomes more efficient through harmonised standards, automation and integration with routine care, improving both data quality and clinician engagement. **[practice change]**.

2.2 Support EHDS readiness [Implementation action]

Challenge: Uneven digital maturity, infrastructure and expertise leave many registries and research networks insufficiently prepared for the technical and organisation requirements of the EHDS.

Recommendation: Introduce an EHDS-readiness assessment for rare disease registries and clinical research networks, covering technical, organisational, legal and financial readiness (including checklists[1]), and provide targeted support where gaps are identified. This should include readiness to interact with HDABs and should be accessible to patient organisation-led registries.

Responsible actors: ERDERA support structures (such as Data Service Hub), research funders, research-performing organisations including hospitals/ERNs, and relevant national EHDS bodies/HDABs.

Implementation lever: National EHDS implementation plans, HealthData@EU deployment, ERDERA onboarding support, EOSC, ELIXIR.

Future state: Rare disease registries are prepared to participate effectively in the EHDS, including meeting its technical, organisational, legal and financial requirements and interacting with HDABs **[system change]**.

1 (see e.g. EHDS readiness guide), https://saludcomply.com/en/ehds/compliance-checklist/

2.3 Adopt common European data models [Implementation action]

Challenge: Registries use inconsistent terminology, coding systems and data structures across countries and disease areas, limiting semantic and syntactic interoperability and the ability to combine and reuse data. Standards and policy frameworks largely exist, but inconsistent implementation and insufficient guidance on how different standards should be used together limit their impact.

Recommendation: Require and fund the appropriate and consistent use of fit-for-purpose common data models, terminologies and exchange standards, with clear guidance on their intended use and interoperability, including OMOP for observational data analysis and HL7 FHIR for data exchange. Support implementation through stewardship and decision-support tools, and ensure alignment with the EEHRxF and, as they become available, relevant JARDIN recommendations. For rare disease identification, require ORPHAcodes to be captured at the point of care and maintained throughout subsequent data flows, using standardised, curated mappings where ICD, SNOMED CT or other coding systems are used alongside them.

Responsible actors: European Commission, JRC, standards bodies, research funders, research/health communities.

Implementation lever: EHDS/EEHRxF; ERDERA and JRC/EU Rare Disease Platform guidance and tools; relevant standards and implementation guides; FP10 funding conditions, European Competitiveness Fund.

Future state: Rare disease registries use interoperable data models that enable data to be combined, compared and reused across diseases, countries and research infrastructures while remaining compatible with international standards. **[system change]**.

2.4 Scale federated FAIR data access [Implementation action]

Challenge: Fragmented platforms, uneven FAIR implementation and legal and technical barriers make it difficult to discover, access and analyse registry data across borders.

Recommendation: research funders (including ERDERA) must publish a conformance profile requiring every funded registry to expose a documented Application Based Interface (API), record-level output in HL7 FHIR, and machine-readable discovery metadata — whatever the internal storage model. Discovery metadata should provide sufficient information to assess fitness for purpose before an access request, including the population, coverage, completeness, coding, granularity, linkage potential and key limitations of the dataset. Support privacy-preserving linkage across datasets and countries where this is necessary to assemble viable rare disease populations and longitudinal evidence.

Responsible actors: Research funders, including ERDERA.

Implementation lever: RD registries, ERDERA Data Service Hub, HealthData@EU and EOSC interoperability framework.

Future state: Researchers discover and analyse distributed rare disease datasets through federated access mechanisms, without unnecessary duplication of sensitive data and without custom integration for each registry. **[system change]**.

2.5 Harmonise legal collaboration frameworks [Enabler]



Challenge: Divergent institutional legal approaches and late involvement of legal teams can delay cross-border collaboration and data sharing.



Recommendation: Develop common European templates and practical guidance for cross-border research collaboration, including model collaboration and data-processing agreements, and involve legal teams from the outset of projects. (see also IHI Data Sharing Playbook and forthcoming JARDIN recommendations on common templates and legal toolbox).



Responsible actors: European Partnerships and legal teams from involved institutions, and patient organisation umbrella bodies.



Implementation lever: ERA Policy Agenda, model collaboration agreements, EC guidance on cross-border research.



Future state: Cross-border collaborations operate within common legal principles and standardised collaboration agreements, reducing administrative burden and accelerating multinational research. **[system change]**.

Theme 3 Align funding incentives

Strategic objective: align funding, evaluation and research assessment with long-term data quality and reuse.

3.1 Embed minimum data requirements and reward data stewardship in funding [Policy reform]



Challenge: Research funding programmes often lack clear minimum requirements for data quality, standardization, preparedness for the emerging EHDS framework, regulatory planning and advice, as well as future data usability. At the same time, funding incentives continue to prioritize project delivery over data quality, interoperability, and long-term data reuse, leaving critical aspects such as data stewardship and regulatory strategy inadequately recognized and rewarded.



Recommendation: Restructure funding mechanism by requiring funding calls to include minimum core requirements for data standardization, EHDS alignment, long-term usability regulatory readiness, mechanisms to monitor compliance and quality and patient and community involvement in data governance, and in line with FAIR principles and appropriate principles for responsible and participatory data governance, while actively rewarding data quality through project milestones and evaluation criteria. Ensure that research assessment frameworks recognise data sharing, curation, interoperability, reuse and patient partnership as formal research impact in grant evaluation and career assessment. Introduce funding eligibility for data stewardship, interoperability compliance, regulatory strategy and patient organisation participation to support readiness for regulatory submissions and evidence generation.



Responsible actors: European Commission, national research funders, and European institutions with authority to legislate, regulate, coordinate, or fund.



implementation levers: FP10, national funding programmes.



Future state: Publicly funded research consistently generates high-quality, interoperable datasets that are fit for future regulatory use and end-to-end evidence generation strategies, as core data stewardship requirements become a condition of funding. As a result, research outputs are better positioned to progress from the laboratory to real-world regulatory assessment, while long-term data reuse, data curation, and regulatory compliance are recognised as valuable research outcomes. **[policy]**.

Theme 4 Patient partnership in rare disease data

Strategic objective: recognise patients and patient organisations as partners in the rare disease data they help generate.

4.1 Recognise patient organisations as contributors to research output [Policy reform]



Challenge: Patient organisations are consulted as stakeholders rather than recognised as partners in the registries their communities populate.



Recommendation: Where a patient organisation provides essential resources — samples, recruitment, cohort or registry data — the project must offer participation in interpreting results and revising the manuscript, and name the patient organisation as author where that participation occurs. Acknowledgement alone is not sufficient. The organisation must receive the manuscript 15 working days before submission, with the right to comment, not to veto.



Responsible actors: European Commission (DG RTD); Supporting: ERDERA, national funders.



Implementation lever: FP10 model grant agreement (dissemination obligations); ERDERA call conditions; ICMJE authorship criteria.



Future state: Patient organisations whose contribution makes research possible appear in the record as contributors and see results concerning their own community before publication. **[policy]**.

4.2 Recognise patient organisations as data users on equal terms [Implementation action]



Challenge: Patient organisations are not recognised as data users on equal terms, and cannot discover, access and analyse registry data on the same basis as researchers.



Recommendation: Patient organisations must be eligible data requesters on the same terms as academic applicants: same process, same criteria, no aggregate-only tier. Conformance must be testable and required by funders.



Responsible actors: ERDERA; Supporting: research funders.



Implementation lever: HealthData@EU.



Future state: A patient organisation can query data on its own disease on the same terms as a research performing organisation. **[system change]**.

4.3 Return results to participating families [Implementation action]

Challenge: Families contributing data do not consistently receive meaningful feedback on how their contribution has been used or what the research has generated; the resulting attrition degrades the longitudinal completeness that makes rare disease registries valuable for regulatory and HTA use.

Recommendation: Require return-of-results and feedback pathways as an expected element of Data Management Plans and an eligible cost in funding calls, co-designed with patient organisations.

Responsible actors: ERDERA; national funders; national health authorities; registry owners; ERNs.

Implementation lever: ERDERA call conditions and DMP requirements; FP10 eligible costs; EHDS transparency obligations.

Future state: Families receive meaningful feedback on their contribution, sustaining participation, improving data completeness and strengthening the social licence for secondary use. **[practice change]**

4.4 Co-design patient-relevant outcomes and data collection with patient organisations [Implementation action]

Challenge: Study designs and data collection do not consistently reflect the outcomes that matter to patients, reducing the relevance of the resulting evidence for regulatory, HTA and healthcare decisions and diminishing the value of the data patients help generate.

Recommendation: For projects generating clinical or outcome data, require patient-relevant outcomes to be defined with patient organisations where relevant to the study and its intended use, and evaluate this explicitly in funding calls. Ensure that early regulatory and methodological support incorporates patient priorities, so that evidence-generation plans align patient, regulatory and HTA needs from the outset.

Responsible actors: Research funders (incl. ERDERA); EMA; national HTA bodies; patient organisations.

Implementation lever: FP10 Rules for Participation and evaluation criteria; ERDERA call conditions; EMA Scientific Advice and European/national HTA advice mechanisms.

Future state: Patient priorities are incorporated into study design and evidence generation from the outset, improving the relevance of resulting data for patients, regulators, HTA bodies and healthcare decision-makers. **[practice change]**.

4.5 Fund and reward patient organisation participation across the data lifecycle [Policy reform]

Challenge: Patient participation, the governance work patient organisations perform, and the return of results to families are treated as unfunded expectations rather than legitimate, eligible research costs, and are not consistently rewarded in evaluation.

Recommendation: Introduce funding eligibility for patient organisation participation — including data governance, co-design and return-of-results activities — alongside data stewardship, interoperability compliance and regulatory strategy. Make patient and community involvement a minimum core requirement of funding calls and actively reward it through project milestones, evaluation criteria and research assessment. Support visibility through case studies and best practices, including examples from registries established and governed by patient organisations.

Responsible actors: European Commission; national research funders; ERDERA; patient organisation umbrella bodies.

Implementation lever: FP10 legislative proposal and Work Programmes; ERDERA call conditions and proposal evaluation criteria; CoARA implementation; EURORDIS blueprint; ERDERA Knowledge Hub / Virtual Platform for case studies.

Future state: Patient organisation participation across the data lifecycle is appropriately resourced, recognised and rewarded as an integral part of high-quality rare disease research. **[policy]**.

Annex A Implementation Roadmap

Theme / recommendation	Type	Implementation action	Responsible actors	Primary implementation lever	Actions for ERDERA
1.1 Regulatory readiness in funding calls	Policy reform	Require regulatory readiness in funding calls	EC, ERDERA, national funders	FP10/Horizon Europe Work Programmes; ERDERA call conditions and evaluation criteria	Embed regulatory-readiness, data-quality and reuse requirements in ERDERA calls and evaluation criteria; require appropriate regulatory expertise in funded projects.
1.2 Early regulatory support	Enabler	Provide early and lifecycle regulatory/ HTA support	ERDERA, EMA, HTA bodies	ERDERA regulatory support services; EMA Scientific Advice; HTA advice	Provide/facilitate access to regulatory support from proposal development onwards and help projects use relevant EMA/HTA advice pathways.
1.3 Regulatory case studies	Enabler	Develop and disseminate case studies on successful use of registry/RWD	ERDERA, EMA	ERDERA Data Hub/Virtual Platform; DARWIN EU; ERDRI	Compile and publish practical examples of registry and RWD use in regulatory, HTA and healthcare decision-making, including patient-led registries.
1.4 Harmonised GDPR guidance	Enabler	Promote consistent application of data-protection requirements	EDPB, EC, Member States	EDPB/EHDS guidance and implementing measures	Identify recurring legal barriers encountered by ERDERA projects and registries and feed implementation experience into relevant EU guidance processes.
2.1 Reduce HCP burden	Implementation action	Fund workforce, tools and automation for point-of-care data capture	National authorities, healthcare providers, EHR vendors, hospital managers	EHDS; national digital-health programmes	Demonstrate and share approaches that reduce data-capture burden and improve integration with routine clinical workflows.
2.2 EHDS readiness	Implementation action	Assess and support registry/network readiness for EHDS	ERDERA, funders, RPOs, HDABs	National EHDS implementation; HealthData@ EU; EOSC/ ELIXIR	Develop/use an EHDS-readiness assessment and provide targeted onboarding support, including for patient organisation-led registries.

Theme / recommendation	Type	Implementation action	Responsible actors	Primary implementation lever	Actions for ERDERA
2.3 Common data models and standards	Implementation action	Require fit-for-purpose, interoperable standards and terminologies	EC, JRC, standards bodies, funders, research/ health communities	EEHRxF; standards and implementation guides; FP10	Provide stewardship, guidance and decision-support tools for appropriate implementation of standards; support consistent ORPHAcode use and mappings.
2.4 Federated FAIR data access	Implementation action	Establish testable interoperability/ discoverability requirements for registries	Research funders, including ERDERA	ERDERA Data Service Hub; HealthData@ EU; EOSC	Define and test conformance requirements for ERDERA-supported registries and enable federated discovery/ access through ERDERA infrastructure.
2.5 Legal collaboration frameworks	Enabler	Develop common templates and guidance for cross-border collaboration	European Partnerships, institutional legal teams, patient organisations	ERA Policy Agenda; model agreements; EC guidance	Develop/adopt model collaboration and data-processing agreements and guidance, drawing on existing tools such as the IHI Data Sharing Playbook.
3.1 Minimum data requirements and stewardship	Policy reform	Embed minimum data requirements and reward stewardship	EC, national funders	FP10; national funding programmes	Embed minimum data-quality, interoperability, reuse and stewardship requirements in ERDERA calls; make associated implementation costs eligible and recognise delivery through milestones/evaluation.
4.1 Patient organisations as research contributors	Policy reform	Recognise substantive patient-organisation contributions to research outputs	EC; ERDERA and national funders	FP10 model grant agreement; ERDERA call conditions; authorship criteria	Set expectations in ERDERA-funded projects for appropriate recognition and involvement of patient organisations in interpretation and dissemination of results.
4.2 Patient organisations as data users	Implementation action	Enable equitable access to registry data	ERDERA, research funders	HealthData@EU	Ensure ERDERA data-access processes allow patient organisations to apply for data access on transparent and equitable terms.

Theme / recommendation	Type	Implementation action	Responsible actors	Primary implementation lever	Actions for ERDERA
4.3 Return results to families	Implementation action	Require return-of-results and feedback pathways	ERDERA, national funders, registry owners, ERNs	ERDERA call/DMP requirements; FP10 eligible costs	Require feedback/ return-of-results planning in relevant ERDERA projects and recognise associated activities as eligible costs.
4.4 Co-design patient-relevant outcomes	Implementation action	Incorporate patient priorities from study design onwards	Funders, ERDERA, EMA, HTA bodies, patient organisations	Funding evaluation criteria; regulatory/ HTA advice	Require and evaluate meaningful patient-organisation involvement in defining patient-relevant outcomes in relevant ERDERA-funded projects.
4.5 Fund and reward patient participation	Policy reform	Resource and reward patient participation across the data lifecycle	EC, national funders, ERDERA, patient organisations	FP10; ERDERA calls/evaluation; CoARA	Make patient-organisation participation, governance and feedback activities eligible costs and recognise meaningful participation in ERDERA evaluation and project milestones.

Annex B Where implementation happens

Implementation arena	Main instruments	Primary owners
EU legislation & implementation	EHDS Regulation; EHDS Implementing Acts; guidance for Health Data Access Bodies	European Commission; Member States
Research funding programmes	FP10 Rules for Participation; Horizon Europe Work Programmes; ERDERA annual calls; proposal evaluation criteria	European Commission; ERDERA; national funders
Research support & capacity building	ERDERA Capacity Building Programme; ERDERA regulatory support service; EMA Scientific Advice	ERDERA; EMA
Technical standards & infrastructure	HL7 FHIR; ORPHAcodes, SNOMED, ICD-11, GA4GH; EEHRxF; HealthData@ EU; ERDERA Virtual Platform; EOSC interoperability framework	European Commission; JRC; standards organisations; ERDERA
Governance & coordination	ERA Policy Agenda; ERN governance; registry governance frameworks; model collaboration agreements; EURORDIS blueprint	European Commission; Member States; ERDERA; ERNs; patient organisations
Research assessment & incentives	CoARA implementation; FP10 evaluation framework; grant reporting requirements	European Commission; research funders; universities

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