

Forum For Exchange of Information

for ERN-Industry collaboration in rare disease research,
powered by Together For Rare Diseases

ERICA ERN Research Conference, Udine, 2024

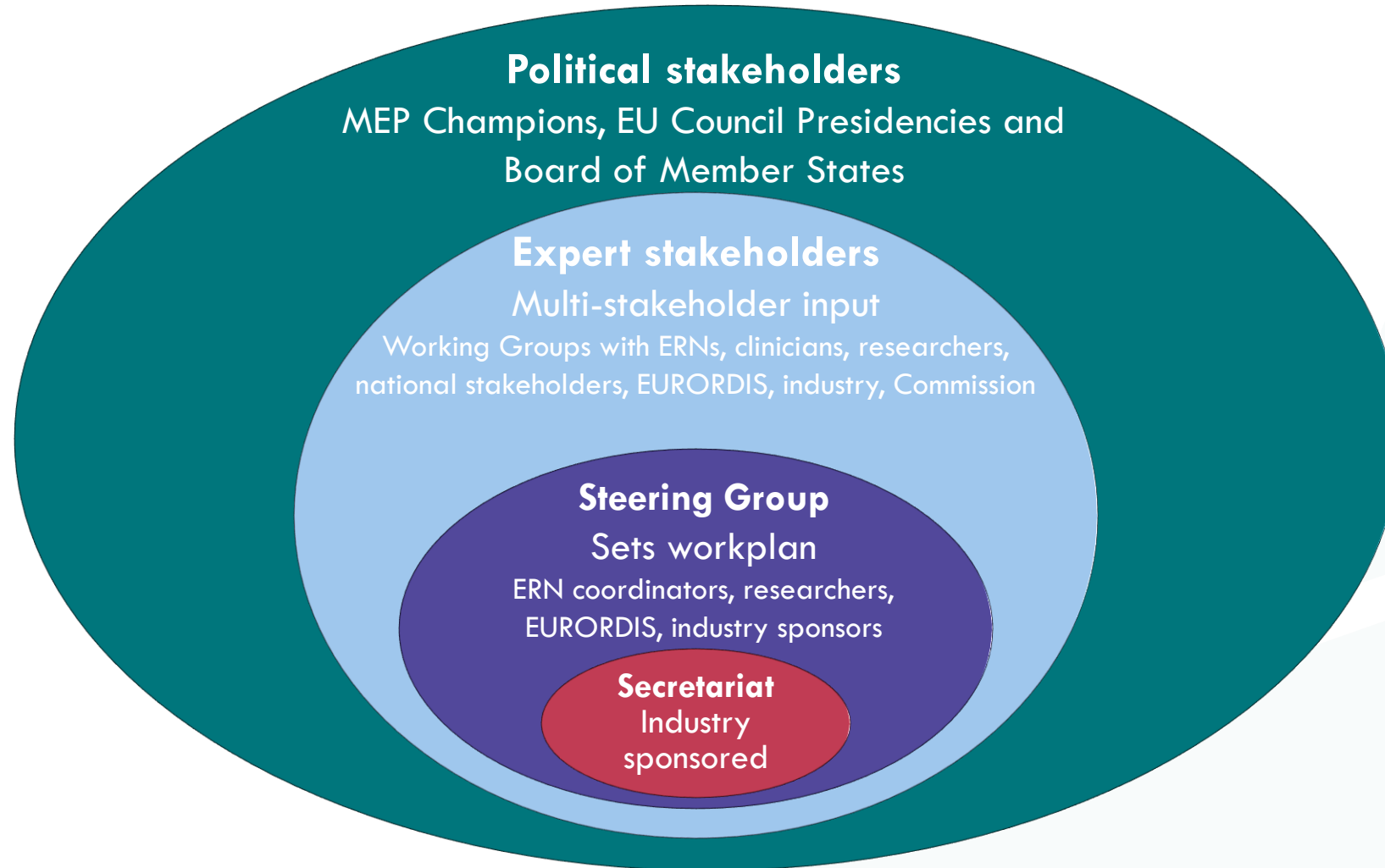


Together for Rare Diseases overview

A Multi-stakeholder Alliance aims to **unlock opportunities for ERN collaboration with the pharmaceutical industry** that:

- Accelerate research to address the 95% of rare diseases without a dedicated treatment
- Promote the EU to global industry leaders as a place for ground-breaking rare diseases research
- Fulfil the potential of ERNs in the research field by developing transferable learning that can benefit all ERNs, no matter their size, resourcing, current intensity of research etc.

Governance & stakeholder engagement



Together4RD Steering Group Members

ERN Coordinators



Hélène Dollfus
(ERN-EYE)



Franz Schaefer
(ERKNet)



Alexis Arzimanoglou
(EpiCare)



Maurizio Scarpa
(MetabERN)



Isabella-Anna Vacchi
(ERN-EYE)

Researcher Community



Daria Julkowska
(ERDERA)



Ana Rath
(Orphanet)



Anton Ussi
(EATRIS)



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(EJP RD)



Rima Nabbout
(EJP RD)

Steering Group Chair



Sheela Upadhyaya
(FIPRA)

Patient Advocacy rep. from EURORDIS



Matt Bolz-
Johnson



Graham
Slater

Industry (Together4RD funding partners)



Toon Digneffe
(Takeda)



Anne-Sophie Chalandon
(Sanofi)



Morgane Cuisenier
(Novo Nordisk)



Gabriella Almberg
(UCB)



Matteo Scarabelli
(EFPIA)



Stefania Alessi
(EUCOPE)

Secretariat
FIPRA



Three pilots

LAUNCHED



**ERN-industry partnership on
Natural history
Innovative digital outcome measures
*Osteogenesis Imperfecta***



ERN BOND
EUROPEAN REFERENCE NETWORK
ON RARE BONE DISEASES

sanofi

LAUNCHED



**ERN-industry partnership on
Unlocking the potential of data in
Rare Renal diseases: RHINE project
*Harmonisation and robust
interoperability model***



ERKNet
The European
Rare Kidney Disease
Reference Network



novo nordisk®

OIXIAL|EUROPE
European Hyperoxaluria Consortium

LAUNCHED



**ERN - industry partnership on
improving diagnostics for
rare hematologic diseases**



ebn
ERN | EuroBloodNet



NOVA
Advanced Diagnostic Platform

enrol

Hedley *et al.*
Orphanet Journal of Rare Diseases (2023) 18:272
<https://doi.org/10.1186/s13023-023-02853-9>


Orphanet Journal of
Rare Diseases

POSITION STATEMENT

Open Access



Together4RD position statement on collaboration between European reference networks and industry

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Remaining gaps

“Many companies [...] are **generally not aware of what ERNs actually offer** nor what they have achieved and what priorities they are embracing for the coming years”

(Together For Rare Diseases Position Paper, 2023)

“PPPs are an ecosystem. In that ecosystem or ‘*community of brains*’, every partner plays a role. In addition to financial resources, **industry brings complementary skills sets, knowledge and competence**. Central to success is a mutual understanding of objectives and the ‘end goal’, and the importance of building and sustaining trust throughout the partnership.”

(IRDIRC project: The different contributions of the industry in public-private partnerships in Rare Diseases Research continuum, 2024)

4 possible models of fora

All ERNs – all companies
Research conference

All ERNs – all companies
Strategy forum

ERN-specific – interested companies
Therapeutic area

ERN sub-network - interested companies
Disease specific

Success factors

- ❖ Focus as specific as possible
- ❖ Right people in the room
- ❖ Incentives for both parties
- ❖ Credibility of the context
- ❖ Clarity on problem statement

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Success factors

- ❖ Focus as specific as possible (therapeutic area – disease specific)
- ❖ **Researchers (academia and private) focusing on the same disease(s)**
- ❖ Incentives for both parties
- ❖ Credibility of the context
- ❖ Clarity on problem statement

Success factors

- ❖ Focus as specific as possible (therapeutic area – disease specific)
- ❖ Researchers (academia and private) focusing on the same disease(s)
- ❖ **Clear description of "what's in it for me?"**
- ❖ Credibility of the context
- ❖ Clarity on problem statement

Success factors

- ❖ Focus as specific as possible (therapeutic area – disease specific)
- ❖ Researchers (academia and private) focusing on the same disease(s)
- ❖ Clear description of "what's in it for me?"
- ❖ **Research congresses and/or patient advocacy group-organised conferences**
- ❖ Clarity on problem statement

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- ❖ **All parties involved need to be clear on the problem statement**

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- ❖ Focus as specific as possible (therapeutic area – disease specific)
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- ❖ Clear description of "what's in it for me?"
- ❖ Research congresses and/or patient advocacy group-organised conferences
- ❖ All parties involved need to be clear on the problem statement
- ❖ **ANY OTHER BRILLIANT IDEAS?**

2 possible models of fora

All ERNs – all companies
Research conference

All ERNs – all companies
Strategy forum

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Therapeutic area

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Disease specific

Let's work together to make it happen

- Identification of opportunities to engage (diseases specific conferences, patient-led, scientific, other...)
- Opening doors
- Research ideas
- Commitment to co-create

What do we (not) bring?

- Manpower and dedication
- No funding

Let's work together

- Identification of research needs
- Opening doors to new ideas
- Research ideas that are feasible
- Commitment to shared goals



(not) bring?
and dedication

Background slides

January 2024

QR code