IHI Call Days | Call 12

NF European Drug Discovery and Development Engine

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Link to the IHI brokerage platform:

- Proposal sharing tool

Challenges and objectives

Neurofibromatoses (NF1, NF2-related schwannomatosis, non-NF2 SWN) are rare, heterogeneous tumor-predisposition syndromes spanning pediatrics and adults. Patient numbers per site are small, phenotypes vary, and *no single country can efficiently deliver adequately powered studies*, which slows therapy development and perpetuates inequities in care access across the EU. Care is patchy: some member states have expert clinics, many do not, resulting in uneven standards and limited research participation.

Fragmentation & EU–US imbalance. Although **many NF drug candidates originate in European laboratories**, early clinical development frequently starts in the US, reflecting Europe's fragmented trial readiness and multi-jurisdictional complexity. This drains opportunities for EU patients and investigators and delays European evidence generation.

IHI alignment. The project addresses IHI's objectives to: (i) reduce fragmentation of R&I across stakeholders; (ii) deploy digital, data and platform approaches to enable **early, robust feasibility** and **multi-country execution** in complex indications; and (iii) improve equity and quality of care for patients with unmet needs through standardized pathways and cross-border collaboration.

Your approach to solve the problem

What we will build (4-year programme following a 1-year map-and-design phase):

- A **pan-EU trial platform** (derived from EU PEARL - an **NF1 adaptive platform master protocol**) capable of multi-arm evaluation with shared controls and rapid arm add/drop.
- A **central coordination hub** with harmonized SOPs, regulatory/ethics support for CTR/CTIS submissions, contract templates, and site-readiness tooling to shorten start-up.
- An **interoperable EU NF registry & data resource** (EHDS, GDPR-compliant), integrated with routine care data (imaging, pathology, genomics) to power feasibility, real-world evidence, and PROs.
- **Care harmonization**: operationalizing ERN guidance into practical care pathways, tele-tumor boards, cross-border referral, and progressive **center accreditation** to level standards across all member states.
- **Capacity-building**: twinning/mentorship, investigator and study-team training, common outcomes and measurements (e.g., volumetrics, audiovestibular metrics, dermatologic burden), and a thriving patient-involvement program.

Early project studies: NF1 platform trial launch; NF2/SWN natural-history study to mature endpoints and site capability for subsequent interventional work.

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Is your project suitable for IHI?

Large-scale, cross-sector innovation. The network is explicitly **public–private** and multi-stakeholder:

- **Pharma/biotech** provide assets, drug supply, translational biology, and co-design of platform arms.
- **Medtech/diagnostics** contribute imaging standardization, quantitative volumetrics, audiology, and device-enabled assessments.
- **Digital/IT** deliver registry/EDC platforms, interoperability, eConsent/ePRO, data governance, and analytics for adaptive decisions.
- **Academia/clinicians** supply trial sites, clinical leadership, and care-pathway implementation across member states.
- **Patients/advocacy** (e.g., CTF Europe and national groups) embed patient priorities into endpoints, consent, burden reduction, and equitable access.
- **Regulators/legal experts** (EMA/NCA liaisons) guide innovative designs and cross-border compliance from the outset.

No single sector or country can deliver this at scale; **IHI funding de-risks the shared infrastructure** (platform protocol, registry, legal toolkits, training) and creates a durable European capability that directly counters today's EU–US development imbalance by making Europe the *easiest* place to run NF trials. This directly operationalizes IHI's objective to integrate fragmented health R&I and to exploit digitalization for multi-country, patient-centred innovation.

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Outcomes and Impact

Results, outcomes and impact:

- Operational, multi-country NF trial platform
- Interoperable EU NF registry & data resource (GDPR/EHDS-aligned)
- Care harmonization at scale
- Trial toolkits & capacity

System-level impact:

- Sustainable non-profit network entity that continuously onboards sites/arms, attracts pipelines to Europe, and embeds the model in EU policy frameworks (ERNs, EHDS). *Impact:* more EU-led trials, shorter start-up times, broader geographic access, and an exportable blueprint for other rare diseases.

The project turns Europe's fragmented NF landscape into a **coordinated, trial-ready and care-ready network**—delivering faster answers for developers, stronger competitiveness for the EU health industry, and **tangible, equitable benefits** for patients and families.

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Expertise and resources

We have:

- **Platform-trial know-how and IMI pedigree.** (EU-PEARL neurofibromatosis work package**,** US- based INTUITT - NF2 adaptive basket/platform trial design experience)
- **Patient-centred data and engagement infrastructure.** (NF Registry)
- **Clinical-care network models and standards.** (CTF's NF Clinic Network).
- **Regulatory, ethics and start-up capabilities.**
- **European footprint and convening power.**
- **Pre-IHI head start** (clinical trial and care capacity mapping)**.**

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Expertise and resources

We are looking for:

- **Pharma/biotech:** NF-relevant assets, drug supply, translational biology, regulatory expertise and co-design of platform arms.
- **Medtech/diagnostics:** NF-relevant therapies, contribution in endpoint development, device-enabled assessments.
- **Digital/IT:** registry/EDC platforms, interoperability, data governance and analytics for adaptive decisions.

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