FIELD MANUAL ADDENDUM

FROM PROJECTS TO IMPACT:

Lessons from IHI Public-Private Partnerships Supporting the Rare Disease Ecosystem



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EXECUTIVE SUMMARY

For over fifteen years, the Innovative Medicines Initiative (IMI) and its successor, the Innovative Health Initiative (IHI), have supported a wide portfolio of public–private partnerships (PPPs) designed to accelerate biomedical research, respond to current health needs and foster cross-sector collaboration. While many projects have generated valuable scientific knowledge, platforms, and tools, the long-term implementation and impact of these assets is difficult to assess.

Although there has been a noticeable increase in focus and interest on impact across Horizon Europe, reflected in both new reporting requirements and the growing availability of tools designed to support asset exploitation, it is unclear whether these resources are being used by IHI consortia.

This report is the output of an EFPIA-EURORDIS initiative aiming at optimising IMI/IHI project outputs to boost real-world impact within the rare disease research ecosystem. The analysis draws from a scan of past IMI/IHI projects, 31 semi-structured interviews with project coordinators and partners and a multi-stakeholder workshop held in September 2025. Four case-studies, those of EU-PEARL, c4c (conect4children), WEB-RADR, and AIMS-2-TRIALS, provide examples of different project pathways and demonstrate cross-cutting lessons for future projects.

A steering committee (SC) made up of EFPIA representatives had regular updates and provided input throughout the project.

Overall, several enabling factors emerged consistently across projects:

- **Including implementability in project planning.** Striking the right balance between novelty and usability can increase real-world impact;
- **Involving the right stakeholders** at the right time and in the right way, including:
  - **Patient and end-user involvement** that can directly shape the scientific direction and increased relevance and uptake;
  - **Cross-sector collaboration**, including regulators.
- **Early planning for post-project future and deployment**. This includes clear governance and ownership models for post-project assets;
- **Targeted communication and training strategies** focused on uptake rather than passive dissemination.

Certain recurring barriers also became evident, such as: limited long-term funding mechanisms, a lack of know-how in developing post-project deployment strategies and misaligned stakeholder incentives.

Based on these findings and building directly on the workshop's conclusions, this report provides *project-level recommendations* to help consortia plan for impact and deployment, as well as *framework-level recommendations* to strengthen structural support within IHI. While developed through a rare disease lens, the lessons and recommendations presented here are widely transferable across disease areas and research domains. They underline a core message shared across interviews and the workshop: that impact considerations must be designed into the project and then subsequently supported and stewarded beyond the lifetime of the grant.

FROM PROJECT OUTPUT TO IMPACT IN MEDICAL AND CLINICAL PRACTICE

A STARTER CHECKLIST FOR IMI/IHI CONSORTIA

Five takeaways at a glance

1. Balancing novelty with usability leads to actionable assets that are more readily validated, adopted and sustained in real-world settings
2. Uptake depends on engaging the right stakeholders early and meaningfully, including regulators where relevant, patients and other end-users/implementers so that outputs align with real-world needs, and build ownership and demand during the project.
3. Post-project ownership, governance and hosting must be addressed early (ideally by mid-project) to avoid an end-of-grant "cliff edge".
4. Training, onboarding and practical materials are often the missing link between dissemination and genuine uptake.
5. At the programme level, the IHI framework could potentially be more involved in the post-project trajectory of assets by increasing emphasis on impact and uptake, and backing this with additional dedicated funding and structural support.

Use this check list at proposal stage — and revisit it regularly during the project.

It complements the Field Manual on Scaling Innovations from Public–Private Partnerships: use this page to make sure the conditions for uptake are being built into the project, then use the Field Manual to develop the scale-up pathway.

1. START WITH THE USE — NOT JUST THE OUTPUT

- [ ] Can we describe what will be different in practice if this project succeeds?
- [ ] Have we identified the specific asset(s) we expect others to use — tool, platform, method, standard, dataset, network, service, etc.?
- [ ] Do we know who should use it, where, and for what purpose?
- [ ] Are our success measures about use/uptake as well as scientific delivery?
- [ ] Are we prepared to prioritise a usable and implementable solution over a more novel but difficult-to-adopt one?

Reality check → **Could a prospective user explain why they would use this?**

EXAMPLE

AIMS-2-TRIALS: the consortium accepted a more modest biomarker outcome but focused on a regulatory validation pathway, making the result more usable than a novel but unqualified biomarker. See case study / findings on implementability.

2. GET THE RIGHT PEOPLE AROUND THE TABLE

- [ ] Have we identified the people who will use, adopt, approve, fund or maintain the asset?
- [ ] Are patients and relevant end-users involved in shaping it, rather than only reviewing it?
- [ ] Where relevant, have regulators / HTA bodies / payers / policymakers been engaged early enough to influence development?
- [ ] Are the people representing organisations in the consortium connected to the actual decision-makers needed for later uptake?
- [ ] Are we testing the asset with prospective users during the project?

Reality check → **Is anyone important to eventual adoption missing?**

EXAMPLE

WEB-RADR: patients shaped the Med Safety App requirements, while EMA, MHRA, WHO and FDA involvement helped ensure the solution fitted regulatory practice. The app was subsequently adopted in 22 countries.

3. DESIGN FOR THE REAL WORLD

- [ ] Have we tested whether the asset fits existing workflows, systems and capabilities?
- [ ] Do we understand the regulatory, data, legal, interoperability or operational requirements that could block implementation?
- [ ] Are we generating the evidence future adopters need to make a decision?
- [ ] Are training, guidance, onboarding or implementation materials being developed alongside the asset?
- [ ] Can the solution work outside the consortium and in different settings/countries, or do we know what must be adapted?

Reality check → **What would stop an organisation using this tomorrow?**

EXAMPLE

EU-PEARL: regulator engagement, industry trial-operation expertise, patient co-design and extensive trial simulations helped create master protocols that could be reused in subsequent platform trials.

4. PLAN WHO CARRIES IT FORWARD **(Do not leave this until the final year.)**

- [ ] By mid-project at the latest, have we started discussing what happens to each key asset after funding ends?
- [ ] Is there a credible owner/steward/host — existing organisation, distributed partnership, new entity or another model?
- [ ] Are ownership, IP, data access, governance and maintenance responsibilities clear?
- [ ] Do we know the resources and capabilities required to keep the asset operational?
- [ ] Have we identified possible future funding/revenue/support mechanisms and any funding gap between project end and deployment?

Reality check → **If the grant stopped tomorrow, who would pick this up?**

EXAMPLE

c4c: the consortium started building its post-project model early and established the conect4children foundation before the IMI grant ended; the network now operates independently with national hubs and active services.

5. BUILD UPTAKE WHILE YOU STILL HAVE A PROJECT

- [ ] Are communication activities targeted at specific prospective users, rather than mainly disseminating results?
- [ ] Are we creating users, trained implementers, champions or ambassadors who can carry the asset forward?
- [ ] Can prospective adopters try, test or use the asset before project end?
- [ ] Are we tracking signals of uptake — use, reuse, adoption, endorsement, investment, integration into practice or policy?
- [ ] Are we adapting the deployment plan as evidence and circumstances change?

Reality check → **Are we creating demand and ownership — or simply awareness?**

EXAMPLE

EHDEN: the project did not rely on dissemination alone; it funded data partners to harmonise their data and trained/certified SMEs to support implementation, creating users and implementation capacity while the project was still running.

6. QUICK CHEAT-SHEET: YOUR FIVE-LINE IMPACT PLAN

Our priority asset is .....

The people/organisations we need to use it are .....

The biggest barrier to their adoption is .....

After the project, the asset will be owned/hosted by .....

The next action we will take to increase uptake is .....

Then go to the Field Manual: use the Pre-scale Checklist → Scale-up Vision → Scale-up Pathway to develop the scaling strategy.

CASE STUDIES IN THE REPORT

EU-PEARL — reusable trial methodology | c4c — sustainable infrastructure | WEB-RADR — adoption through regulators and patients | AIMS-2-TRIALS — implementability and regulatory validation.

1 INTRODUCTION

Through IMI and now IHI, the European Commission and the European Federation of Pharmaceutical Industries and Associations (EFPIA) have co-funded large, multi-year, multi-stakeholder collaborations addressing gaps in drug development, clinical research, data infrastructure, regulatory science, and patient engagement.

For the rare disease community, these initiatives are particularly relevant: rare diseases often lack commercial incentives and research depends on coordinated infrastructure, shared platforms, aligned standards, and meaningful involvement of people living with rare diseases. Despite an increased focus on measuring and optimising impact, there remain question marks on how assets developed through IMI/IHI projects fare once projects end and whether they are adopted into routine practice.

This study was commissioned by EFPIA and undertaken by EURORDIS - Rare Diseases Europe with the following objectives: to optimise IMI/IHI project outputs to boost real-world impact; to demonstrate the value of public-private collaboration including its potential to improve the rare disease patient journey; and to further operationalise the "Field Manual on Scaling Innovations from PPPs", one of the recent tools that have been developed to enhance asset uptake. The project aimed to understand the enabling factors behind successful projects and post-project deployment of assets, and to identify barriers that hinder successful implementation. Ultimately the project offers a list of concrete recommendations for future consortia applying for grants and for IHI as a funding framework.

Report Structure

The report begins with cross-cutting findings from the project scan and interviews. It then presents four case studies, offering differing approaches to project management and post-project vision, with their own cross-cutting lessons. These insights feed into a consolidated set of recommendations, aligned with the September 2025 workshop outcomes.



2 METHODOLOGY

This study employed a mixed-methods approach combining desk research, qualitative interviews, guidance from the project SC and collaborative analysis through a multi-stakeholder workshop (held in September 2025).

2.1 Project Scan

A review was conducted of IMI1, IMI2, and early IHI-funded projects to identify those that developed assets relevant to rare diseases or with potential applicability across disease areas.

The scan included the:

- IHI project database;
- CORDIS EU research results platform;
- internal EURORDIS knowledge of projects;
- individual project websites;
- other public information on projects and deliverables, where available.

A longlist of projects was compiled (see Annex I), covering a broad range of thematic areas such as clinical trial innovation, data platforms, patient engagement, translational science, and regulatory science. The review wasn't confined to rare disease specific projects but examined outputs that could potentially have an impact on the rare disease field.

A follow-up shortlist of 20 high importance project assets (Annex II) was selected with the project SC. These 20 projects were then targeted for in-depth interviews.

2.2 Interviews

Thirty-one semi-structured interviews were conducted with coordinators and partners from shortlisted projects. Interviews followed a standard guide (Annex III) covering:

- Asset description;
- Planning for success;
- Implementation and uptake;
- Patient engagement;
- Post-project plans;
- Dissemination and communication strategies;
- Lessons learned and recommendations.

Interviews provided substantial qualitative data, including authentic quotes which have been used throughout the report.

2.3 Workshop

Findings were consolidated during a one-day workshop on 30 September 2025, involving representatives from IHI, EURORDIS, EFPIA, and four selected IHI projects.

The workshop focused on:

- identifying enabling factors and barriers in the deployment and uptake of IHI project assets;
- highlighting transferable lessons through case study insights;
- co-creating actionable recommendations for future IHI calls and consortia.

All conclusions and recommendations presented in this report are aligned with the outputs of this workshop.

2.4 Case Study Selection

Four projects were selected for detailed analysis due to their strong rare disease relevance and their diversity of focus, outputs and scope:

AIMS-2-TRIALS Development of a multi-disciplinary autism research network. Although autism is not a rare disease, rare diseases are an integral part of AIMS-2-Trials.

Several rare diseases such as Phelan-McDermid syndrome, Fragile X, TSC1, and SHANK-related disorders present an autism phenotype and have been included in the project. In addition more than half the individuals registered on the EAGER registry, developed under AIMS-2-Trials, have a rare disease.

EU-PEARL Development of patient-friendly platform trials to improve efficiency of drug development.

One of the four disease-specific applications focused on in EU-Pearl was the rare disease neurofibromatosis.

c4c (conect4children) Establishment of a sustainable, pan-European paediatric clinical trial network to improve trials and accelerate drug development for children.

C4c focused on paediatric clinical trial improvement which has a large added value for rare diseases as 70% of rare genetic diseases start in childhood, and 3 in 10 children with a rare disease won't live to see their 5th birthday. EURORDIS was an active partner in this project.

WEB-RADR Development of a digital app for the direct reporting of adverse drug reactions.

Real-world drug monitoring is crucial for rare disease populations to spot uncommon or long-term adverse reactions. EURORDIS was an active partner in this project.

3 KEY FINDINGS

Across the interviews several recurring themes emerged regarding the development, and uptake of assets created within IMI/IHI projects. These themes cut across disease areas and project types, illustrating broader lessons for the rare disease ecosystem and for public-private partnerships more generally.

3.1 Including implementability in project design

Getting the right balance between usability and novelty can lead to greater real-world impact. Several interviewees highlighted that while scientific innovation is important, projects often over-engineer novel tools that are difficult to validate, adopt, or sustain in a real-world setting. Assets designed with implementability in mind are more readily taken up by regulators and health systems.

In **AIMS-2-TRIALS**, for example, partners explained that although the project produced a more modest biomarker outcome than initially hoped for, it was nonetheless tailored to regulatory requirements leading to the development of a biomarker development and validation pipeline. "*There are a lot of publications purporting to have found a biomarker, but none of them are validated by international regulators,*" one interviewee noted, underscoring the value of delivering something usable rather than merely novel.

Cancer-ID similarly shifted its focus during the project from broad biomarker discovery towards benchmarking assays and protocols which have since been reused by other consortia. The project's shift towards a more modest yet actionable output demonstrates how practical outputs enable wider uptake.

WEB-RADR offers one of the clearest illustrations of implementability, with assets that were very precise and tangible. "What was really valuable about WEB-Radr is that the outputs weren't just more research questions. They were things you could take and use, like the MedSafety app." (Web-RADR coordinator)

Projects that were consciously designed for implementability by aligning early with user needs, regulatory expectations, and operational realities ultimately delivered assets with stronger pathways to adoption.

3.2. Involving the right stakeholders

Across projects, the involvement of relevant stakeholders was flagged as one of the most important elements of project success.

Regulators and cross-sector collaboration

Almost every coordinator interviewed emphasised the central role of regulators, noting that structured early engagement ensures that outputs reflect real-world constraints and expectations.

- The **AIMS-2-TRIALS** interviews emphasised that different approaches are needed to achieve different project end goals. As one academic partner of the project said: *"If you really go in there with the end goal not being to publish a paper in a high-impact journal, but the end goal of actually delivering something that we can use in clinical trials and clinical practice, it's a very different approach."* (AIMS2Trials academic partner). This shift in mindset towards obtaining clinically usable outputs was credited to the fact that the project was operating within a PPP, which de facto provides interaction with stakeholders that would not usually work with each other. The PPP enabled direct engagement with regulators, which clarified what would be needed for future regulatory use.

- **T2EVOLVE** also shows how regulatory engagement and alignment with other initiatives can turn project outputs into practical policy tools. By engaging EMA's Innovation Task Force from the start of the project and coordinating with projects working on similar issues (such as the EU-funded Join4ATMP), the consortium avoided duplication, built a shared position, and ensured its work reflected real regulatory needs. This collaboration is now developing a concept paper on platform development strategies including recommendations, enabling the outputs to actually inform real-world policy rather than remain an academic exercise.

Not all project consortia however have clarity on how best to involve regulators or on when this should be done. This can lead to missed opportunities and inconsistent regulatory engagement across projects. It should also be noted that not all projects will need to involve regulators, so when, how and if regulators need to be involved should be considered from the start.

Patient and end-user involvement

Structured and meaningful patient engagement helped refine projects' research questions, adapt methodologies, and assist in communicating about outputs. Across multiple interviews, participants noted that patient input was not simply consultative but had changed the course of scientific and strategic decisions, aligning research with community priorities and assisting real-world uptake.

- Patients were instrumental in shaping assets across several projects: **WEB-RADR** incorporated patient input directly into the app design, improving the likelihood of usability. **AIMS-2-TRIALS** reported that the Autism Spectrum Disorder (ASD) community involvement actually shifted the project's scientific focus towards mental health and lived somatic experiences, which were identified by patients as their primary concerns over other attributes. **ARDAT** engaged patients in co-creating educational materials to support communication of complex scientific outputs. **EU-PEARL** involved patients in co-designing platform trial structures.

Other potential end-users and implementers such as clinicians, SMEs, data partners, and service providers are often overlooked but critical. Assets that are tested, used, and promoted during the project with their end users, create demand, skills, and ownership for post-project uptake.

- Project **EHDEN** provides a concrete example of this. EHDEN's "harmonisation fund" provided small grants to external data partners to map their datasets to the OMOP Common Data Model, creating a strong incentive for organisations across Europe to join the network. At the same time, the project built a certified workforce by training SMEs through a freely available curriculum. This dual strategy enabled more than 200 data partners to onboard with the support of over 60 certified SMEs, rapidly expanding the network and promoting real world uptake all while the project was still ongoing.

Not all projects have the willingness or know-how to meaningfully include or support patient engagement into their structure. A lack of clear roles, expectations and structured support can lead to patient involvement being relegated to a box ticking exercise.

Meanwhile, industry engagement was frequently flagged as suffering from frequent turnover: interviewees noted that individuals involved at project start were often no longer present by mid-project. This frequent turnover was seen as disrupting continuity, weakening institutional memory, and slowing project progress. It was also noted that, while industry is involved in these projects, it remains unclear whether information on consortia outputs and final assets is systematically shared or whether it ever reaches internal decision-makers.

3.3 Early planning for post-project future and deployment

Although it is difficult to have an idea of a project's future before it has started, interviews indicated that post-project planning should be initiated early on and well before the completion of a project. The planning should include consideration of ownership models, governance structures and business planning. If post-project arrangements are weak or ambiguous, assets become difficult to maintain and without stewardship can be left to dissipate. Late and contested sustainability discussions can lead to disagreements, loss of trust, and limited post-project activity. Even without disagreements, late planning can blight a project's success by creating funding gaps and leading to a loss of momentum.

In the cases where independent foundations were set up as a post-project model, the time for completing this process seems to have taken up to two years, indicating that planning should begin at the latest mid-project.

Although many consortia consider the creation of independent foundations as a means of continuing their work, this is by no means the only way to ensure that assets are maintained and promoted. A number of alternative models exist and should be explored so that a consortium concludes on the best possible structure for the individual situation.

- **Connect4children** (c4c) set up their foundation two years before the project ended ensuring that they could operate independently after the funding ended.

  "We used IMI as an incubator. We're now independent, not needing seed funding or venture capital." (c4c interviewee);
- Similarly, **EHDEN** began designing its foundation mid-project, allowing the legal entity, governance arrangements, and funding streams to be in place by the end of the grant;
- In contrast, **PARADIGM** chose not to create a new legal entity. This was an early and measured decision on the consortium's part. Its Open Forum has continued successfully with sustained support from former partners.

These examples show that early decisions about ownership, governance, and long-term hosting models are essential for ensuring that assets remain usable beyond the lifetime of a grant. It was however noted that the necessary know-how for the planning and implementation of a post-project strategy is frequently beyond the capabilities of the consortia, making the task even lengthier, cumbersome and prone to error.

3.4 Coherent communication and training strategies

Another strong theme that appeared in interviews was that project communication should also focus on uptake, rather than simply be seen as passive output. The production of training tailored to prospective end-users can increase the demand for and uptake of project assets. The example of EHDEN's harmonisation fund has been described above but other examples were also forthcoming.

- One such example comes from project **EBISC** which not only developed an iPSC bank as its main output, but also produced and shared frameworks for consent, ethics, and IP, and organised trainings and workshops to explain how to use, deposit, and share iPSC lines within appropriate legal and ethical frameworks. As these requirements are complex and vary across countries and projects, many laboratories would otherwise struggle to deposit or access lines correctly. By developing and promoting these additional tools, the project worked to actively increase the usage of its main asset.

Similarly, project **PREFER** originally aimed to train patient organisations to run preference studies themselves in their own disease areas, so that the methodology could be more widely used and less dependent on a single central structure. This plan was eventually scrapped due to a lack of budget, which also demonstrates one of the challenges frequently faced within these consortia. Small scale funding for additional activities such as trainings could boost uptake but are hard to come by.

4 CASE STUDIES

The following four case studies illustrate different models of deployment. Each case study demonstrates practices that can support future projects.

4.1 Case Study: EU-PEARL

Overview

EU-PEARL (EU-Patient-centric Clinical Trial Platforms) sought to develop adaptive, patient-friendly platform trials to improve the efficiency of drug development. Four disease-specific applications were focused on: neurofibromatosis, major depression disorder, tuberculosis (TB), Non-alcoholic steatohepatitis (NASH).

Assets Developed

EU-PEARL's main assets include:

- the platform trial methodology including master protocol templates, statistical tools, trial simulation software, legal frameworks;
- and the four disease specific applications.

Type of asset based on the Field manual:
KNOWLEDGE

Deployment pathway and asset implementation

The consortium took the decision to not set up an independent entity at the end of the project. The assets (master protocols and results) are now hosted on the EATRIS website and are publicly available.

Three out of the four disease areas included in the project have subsequently progressed:

Depression: Wellcome Trust funding, trial has now launched in 6 European countries.

NF1: platform trial ready; EMA positive feedback; fundraising ongoing.

TB: master protocol adopted by TB consortium.

Main enablers for successful asset development and uptake

- Strong engagement of regulators in the project which adds weight and legitimacy to the subsequent results. Future funders are reassured by regulator participation.
- Industry expertise in trial operations and regulatory pathways.
- Patient co-design in trial development (co-design on procedures and acceptability of endpoints).
- Extensive trial simulations added to the appeal of the assets.
- Working in small, focused teams with strong leadership proved more productive than attempts at co-creating everything at a larger level.

Challenges Encountered

- Complexity and size of consortium slowed early progress, this was also exacerbated by the pandemic;
- NF1 was developed as a prototype for rare disease and received limited follow-up interest;
- There was no dedicated mechanism to carry outputs into implementation after the project ended, finding funding to continue the work proved challenging;
- Without a dedicated institutional host there is a risk of the assets being underused.

Transferable Lessons

- Build post-project ownership plans into the project from the start (who maintains assets and with what resources). Planning requires having someone to take ownership of executing the plan.
- Keep consortia manageable; allocating work to small, motivated teams tends to be more effective.
- Ensure projects are user-driven.
- Maintain structured post-project engagement with companies, at the decision-making level.
- Consider mechanisms (extension funds, investor involvement) to bridge the gap between proof-of-concept and sustainable implementation.

4.2 Case Study: conect4children (c4c)

Overview

The Conect4children (c4c) project formed a pan-European paediatric clinical trial network which was created to improve trials and accelerate drug development for children. It currently has 20 national hubs and a large network of over 400 experts, including patients.

Assets Developed

- The paediatric clinical trial network itself, which has now been institutionalised in the c4c stichting (foundation). Main services:
- Expert advice panels (including children, young people and families).
- Clinical trial support (site finding, feasibility, trial support).

Type of asset based on the Field manual:
PLATFORM

Deployment pathway and asset implementation

The network has now been established as a non-profit foundation (stichting) since 2023 and currently has ~10 staff, ~€1m turnover, and active service delivery. Templates and legal frameworks are in place. The current challenge is scaling up.

Main enablers for successful asset development and uptake

- Strong industry buy-in from the start.
- Early focus on deployment: having the opportunity to establishing the c4c foundation during the project provided a much needed boost.
- Patient and young people involvement was integrated into the project from day one.

Challenges Encountered

- Academic.and entrepreneurial mindsets and goals can sometimes be conflicting.
- Legal/contractual bottlenecks (such as clinical trial agreements at site level).
- Difficulty engaging the right decision-makers in pharma, those involved in consortia may not be decision-makers.
- Practical barriers in setting up the foundation. Considerations regarding banking, governance, the legal shape and country the foundation should be based in are all time-consuming activities and require prior knowledge.
- Difficulty finding working capital (no shareholders, no possibility for loans, limited access to venture capital)

Transferable Lessons

- Build sustainability and business planning into the project from the start;
- Involve the true end-users (those with budgets/ decision power) early to boost subsequent uptake;
- Consider a range of sources for working capital;
- Recognise differences and need for flexibility: moving from an academic mindset to entrepreneurial management can be difficult.

4.3 Case Study: WEB-RADR

Overview

WEB-RADR (Web-Recognising Adverse Drug Reactions) explored how mobile technologies and social media can support pharmacovigilance. Conducted in two phases, it brought together regulators, industry, academia, and patient groups to assess digital tools for safety monitoring and developed the Med Safety App: a mobile tool for patients/ to report adverse drug reactions directly to regulators.

Assets Developed

Key assets included:

- A mobile app for patient reporting of adverse drug reactions (ADRs).
- An ecosystem of APIs enabling integration with national systems.
- Policy recommendations for use of digital tools in pharmacovigilance.

Type of asset based on the Field manual:
PRODUCT

Deployment pathway and asset implementation

Rather than establishing a new organisation, the UK MHRA assumed stewardship of the mobile app platform.

The mobile app:

- Has been adopted by 22 countries;
- Continues to be developed through external grants (such as the Gates Foundation);
- Is integrated into WHO UMC;
- Currently being integrated into the UK NHS app.

Main enablers for successful asset development and uptake

- Strong regulatory involvement from EMA, MHRA, WHO, FDA.
- Patient engagement (patients themselves defined their needs and requirements for a mobile app increasing their subsequent buy-in).
- Funding from Gates Foundation allows low/no-cost access for LLMICs.
- Sustainability focus built in from the start.

Challenges Encountered

- Enabling app to be used in a clinical setting is too complex due to data governance and confidentiality issues.
- There is interest from industry but industry uptake is also complicated, due to conflict of interest considerations.
- Diverse country requirements made customisation complicated, especially in the early phase. This is currently more standardised but the app has not been taken up in Europe for instance due to different requirements.

Transferable Lessons

- Involve regulators from the outset; co-developing guidance avoids pushback later.
- Sustainability and uptake are stronger when tools are hosted by trusted (public) bodies.
- Patient engagement shapes outputs that people are more likely to use.
- Practical and straightforward recommendations/ outputs are important for uptake.

4.4 Case Study: AIMS-2-TRIALS

Overview

AIMS-2-TRIALS is a multi-disciplinary research network focused on autism spectrum disorder (ASD). It is notable for its extensive and structured patient engagement model, involving 42 "A-Reps" (autism representatives), including autistic adults, caregivers, and parents from multiple countries.

Assets Developed

- A European platform of longitudinal cohorts.
- A biomarker identification and validation pipeline developed with the engagement of EMA and FDA.
- A European network of clinical trial sites.
- The EAGER registry of sequenced individuals with consent to recontact (with more than half being people living with a rare disease).

Type of asset based on the Field manual: Platform & KNOWLEDGE

Asset implementation status

The project is currently ongoing until end 2026. The asset implementation status so far is as follows:

- Cohorts ongoing, registry hosted at Elixir Luxembourg for long-term sustainability and usage by others outside the consortium.
- One biomarker candidate progressing as an exploratory endpoint with EMA and FDA.
- Clinical trial sites operational, with harmonised protocols and trial-readiness standards.
- Several trials conducted: such as SHANK3/ Phelan-McDermid

Post-project plans

AIMS-2-TRIALS does not envisage creating a formal organisation but is continuing the work and promoting produced assets in the following ways :

- Integration into existing academic networks.
- Continued collaboration among partner institutions.
- Maintaining strong collaboration with patient communities.
- Distributed ownership rather than a single entity.

Main enablers for successful asset development and uptake

- Strong industry and academic collaboration.
- Regulatory engagement (EMA qualification advice, FDA input) guiding design and validation.
- Patient community involvement in shaping research priorities.
- Collaborative leadership fostering cross-European alignment

Challenges Encountered

- Heterogeneity of autism made it difficult to identify strong biomarkers; the outcomes were more modest than expected.
- Academic incentive structures focus on publishing a lot and quickly which conflicts with the slower, replication-heavy work needed for regulatory qualification.
- Similarly, there is a funding imbalance, a lot of investment goes into cohort building and data collection rather than data analysis and validation. There is little funding available for the replication work.
- Maintenance of large-scale registries are particularly costly. Without clear long-term funding and governance models, the registries will not be sustainable.

Transferable Lessons

- Sustainability must be planned from the start (legal/data hosting solutions).
- Focus on qualification pathways, not just publication. Engage regulators early.
- Invest in meaningful community involvement, co-production shifts research focus to real needs.
- Flexibility is essential. Projects need to be able to adapt to unexpected changes.

5 RECOMMENDATIONS

5.1 Project-Level Recommendations

1. Plan for impact early by defining success criteria and identifying end-users at the proposal stage.
   Defining end-users, enablers, and measures of success early helps align consortium expectations and ensures deliverables are relevant to real-world needs. This approach complements the Field Manual's emphasis on scalability and fosters clarity around the project's long-term purpose.

2. Identify and engage relevant stakeholders at the right time and in the right way: regulatory bodies, patients, end-users
   Embedding experts can ensure that projects identify the right stakeholders and strategies to develop clear, actionable and implementable deliverables. This enhances credibility and facilitates endorsement. Some projects lack familiarity with regulatory, HTA, and policy engagement and could benefit from more structured training on engagement.

3. Design projects for implementation and usability, not just novelty and innovation
   The value of IHI projects lies not only in innovation but also in their practical deployment. By prioritising usability, projects can create outputs that are more readily adopted by end-users. In certain cases, choosing to proceed with more modest outputs that are nonetheless realistic is a better strategy than attempting something novel. Integrating training activities, and the development of guidelines into the project itself, strengthens the subsequent usability of assets.

4. Invest in community building and project ambassadors
   Involving stakeholders and end-users, not only works to co-create more usable products, it also works as a community building exercise that will establish ambassadors for the post-project period. Community building within and beyond consortia is essential to sustain engagement and promote adoption. Establishing project ambassadors and visibility strategies increases awareness and fosters trust among end-users and decision-makers, supporting post-project continuity

5. Examine post-project structure and ownership models early in the project life cycle
   The effort and complexity of developing a post-project deployment model can often be overlooked. Although it is not feasible to have a fully developed proposal at the time of the project call, this process should start at least halfway through a project to provide ample time to complete the planning and elements that are needed (governance structure, ownership etc)

5.2 Framework-Level Recommendations

Strengthen impact components within the current project structure: These measures build on the existing IHI framework by embedding stronger impact expectations directly into project design and evaluation.

6. Introduce mechanisms to better align incentives through evaluation criteria valuing uptake and impact
   Evaluation and funding criteria should value real-world impact and uptake alongside scientific excellence. Encouraging alignment of academic, industrial, and policy incentives would reduce fragmentation and promote a shared focus on deployability. Projects demonstrating verified uptake of tools, platforms, or data could be formally recognised through a visibility mechanism such as an "IHI Impact Badge" or equivalent, reinforcing the value of implementation.

7. Introduce an Impact Work Package in calls to ensure deployment is funded and assessed.
   Including an Impact Work Package within project calls would institutionalise the requirement to plan for deployment, ensuring it receives adequate attention and resources. This would balance the focus on scientific outputs with the need for tangible, real-world outcomes. As organisations specialising in implementation and sustainability typically operate on a full-cost recovery basis, whereas academic teams often under-price themselves in proposals, there would need to be some mitigating actions to avoid an unintended diversion of resources away from core academic work. Mitigating measures could include targeted in-kind contributions from industry partners and the pooling of resources across projects to achieve economies of scale.

8. Introduce a requirement to engage relevant regulatory and policy stakeholders in specific IHI calls where such engagement would have a direct impact on deployment and uptake.
   Projects addressing areas where regulatory, HTA, or policy frameworks are key enablers of adoption should be required to engage the relevant bodies. This would ensure that outputs are designed with implementability in mind, increasing the likelihood of real-world uptake. Criteria could be developed to identify which call topics would need mandatory regulatory involvement.

Examine strengthening impact through establishing separate dedicated structures within IHI: These measures propose creating new specific support mechanisms within IHI to help consortia move from research to uptake.

9. Bridging fund mechanism
   Create a mechanism that can provide seed money for bridging elements and small-scale post-project activities that could increase impact.

10. Knowledge and implementation support
    Create a dedicated IHI Valorisation Office that could provide targeted guidance for sustainability planning, connect projects to potential hosts, and ensure good practices are shared across consortia. This central function would reduce duplication, enhance efficiency, and ensure consistent follow-up on project results. The Valorisation Office would combine: business incubation, coaching and capacity-building, and management of a central repository of project outputs.

11. Examine launching a dedicated IHI call focused on implementation and impact (based on the EnSO model
    A dedicated call could pilot an IHI funding mechanism focused on the deployment and adoption of validated project assets. Such a call would support consortia to scale proven tools, frameworks, or platforms into real-world health settings. This would help bridge the current gap between project completion and long-term impact, testing a new IHI instrument for implementation and cultural change.

6 CONCLUSIONS

Across the interviews and case studies in this report, a consistent message emerged, producing assets is rarely the limiting factor, but maintaining them and ensuring uptake is. Too many outputs remain vulnerable to gradual degradation once a grant ends, even when their value is strong.

A lot is expected of consortia. They are asked not only to deliver ambitious research, but also to anticipate real-world implementation pathways, establish governance and ownership, navigate regulatory and policy interfaces where relevant, build communities of practice, communicate to diverse end-users, and secure post-project resources often without sufficient time, expertise, incentives, or continuity of personnel to do so reliably. In practice, many consortia are not set up to function as long-term stewards of infrastructure, services, or platforms. Where successful deployment did occur, it was typically because sustainability was planned early and a credible host could assume responsibility.

These findings point to an overarching conclusion, that impact needs to be treated as an integral part of the project and beyond, not as an end-of-grant aspiration. Consortia should continue to plan for implementability and uptake from the outset, but IHI can do more to ensure that valuable assets do not "fall off a cliff" at project end. This includes strengthening expectations and incentives for uptake, supporting practical capacity-building and, most importantly creating concrete framework-level mechanisms that steward outputs beyond the grant period. Options set out in this report are first suggestions as to how these could be achieved. They however be seen as part of a larger discussion of how the IHI infrastructure could evolve to secure the uptake of high-quality outputs, and subsequently improve outcomes for patients.

While developed through a rare disease lens, the lessons here are widely transferable. Rare diseases however are even more vulnerable to this implementation gap because of small populations, dispersed expertise, and limited commercial incentives.

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Authorship statement
The author was responsible for the desk research, interviews, analysis and drafting of the report with supervision, input and guidance from the project's Steering Committee (SC).

Acknowledgements
The author gratefully acknowledges the 31 representatives of IMI/IHI projects who participated in interviews and contributed their experience and perspectives to this study. Particular thanks are extended to the representatives of the four projects selected as case studies (AIMS-2-TRIALS, EU-Pearl, conect4children, WEB-RADR), who also took part in the September 2025 multi-stakeholder workshop. Interviewees are not named to preserve confidentiality.

The author would like to thank the project's Steering Committee (composed of EFPIA representatives) for their guidance and feedback during the project.

Particular thanks are also due to Magda Chlebus, Executive Director, Science Policy & Regulatory Affairs, EFPIA, and Nathalie Seigneuret, Senior Scientific Project Manager, IHI (Innovative Health Initiative), whose support and guidance were instumental in bringing this project to completion.

Participation in the interviews, workshop or steering committee does not imply endorsement of the report's findings or recommendations.

October 2026