

Topic idea submitted to IHI - Reference Number: TI_001181

Are you submitting the idea:

- ☐ in your personal capacity?
☒ on behalf of an organisation?

Please select from the list below the type of stakeholders your organization represents:

Working Group containing regulators, companies, academics, patients, and healthcare professionals

1 Title of your idea

Please provide a short title that accurately reflects the objective(s) of your idea:

A supportive framework for the introduction of medical technology for prevention, diagnosis, treatment and management of rare diseases

2 Scope

Explain the specific challenges/problems to be addressed by your idea and how these affect relevant stakeholders, taking into account what is already known and/or available in the field:

Traditionally, rare disease research and development has primarily focused on medicinal products, while the development of medical technologies for rare diseases, orphan devices have been underserved. Nevertheless, many rare disease patients and their carers need medical devices for their prevention, diagnosis, treatment, or care. Few medical technologies are developed specifically for rare disease patients. At the same time, different studies show that over 90% of healthcare practitioners (HCPs) and patients indicate a strong unmet need for new, specific orphan devices. In addition, many healthcare professionals use different devices off-label to respond to this unmet need.

The development of medical technologies for rare diseases faces many of the same challenges as developing orphan medicinal products, such as disease heterogeneity and geographic dispersion. Some geographies have developed supportive specific orphan-device legislation, and other regions, such as the European Union, currently investigate the potential for such incentives. However, while incentivizing orphan device development is essential, many developers also state the need for a better understanding of the evidence needed to inform decision-making and develop guidance on the market access, reimbursement, materiovigilance and administration of these orphan devices. Specifically for rare diseases, this process should consider medical, economic, organisational, social and ethical considerations, as well as the orphan devices, such as the requirement of software updates and connected costs. As such, we think a specific evaluation framework should be developed for medical technologies for rare diseases.

Please indicate which IHI specific objective(s) (SO), as described in the IHI Strategic Research and Innovation Agenda (SRIA), your idea addresses:

["SO3: demonstrate the feasibility of people-centered, integrate health care solutions"
"SO5: enable the development of new and improved evaluation methodologies and models for a comprehensive assessment of the added value of innovative and integrated health care solutions"]

Please select the keywords that are most relevant to your idea:

["Communicable diseases"
"Non-communicable diseases"
"Rare diseases"
"Prediction"
"Prevention"
"Detection"
"Diagnosis"
"Interception"
"Treatment"
"Disease management"
"Digital health"
"Health technology"]

In alignment with the IHI specific objective(s) selected above, specify the objectives of your idea:

- Demonstrate the feasibility of supporting the development and introduction of medical technologies for the prediction, prevention, detection, diagnosis, interception, treatment, and management of rare diseases.
- Develop improved evaluation methods, including materiovigilance studies, for a comprehensive assessment of the added value of medical technologies, specific for rare disease patients
- Develop sustainable and responsible economic business models for medical technologies specific to benefit rare disease patients, to bring and keep these on the market sustainably

3 Expected impacts to be achieved by your idea

Briefly describe the expected impacts to be achieved by your idea, ensuring that they contribute to IHI general and relevant specific objectives, as described in the IHI SRIA:

Impacts are wider long-term effects on society (including the environment), the economy and science, enabled by the outcomes of R&I investments. Impacts generally occur sometime after the end of the project, e.g. successful implementation of digital solutions supporting people-centred care.

***IHI general objectives:** 1. contribute towards the creation of an EU-wide health research and innovation ecosystem that facilitates translation of scientific knowledge into innovations, notably by launching at least 30 large-scale, cross-sectoral projects, focussing on health innovations; 2. foster the development of safe, effective, people-centred and cost-effective innovations that respond to strategic unmet public health needs, by exhibiting, in at least 5 examples, the feasibility of integrating health care products or services, with demonstrated suitability for uptake by health care systems. The related projects should address the prevention, diagnosis, treatment and/or management of diseases affecting the EU population, including contribution to 'Europe's Beating Cancer Plan'; 3. drive cross-sectoral health innovation for a globally competitive European health industry and contribute to reaching the objectives of the new Industrial Strategy for Europe and the Pharmaceutical Strategy for Europe.*

By being able to understand the evidence needed for this specific and innovative class of medical technologies, being medical technologies specific for rare diseases, and which business models to use to bring them to the market sustainably, an increasing amount of approved, suitable medical technologies can be developed, in such a way that they can stay sustainably so on the market. As such more patients can receive a diagnosis, detection, prevention, treatment or management specific to them. This way, we can increase the number of diseases currently treatable by medical technology (currently, only about 1% of rare diseases have a medical technology available for their authorized treatment).

4 Why should your idea become an IHI call topic?

Explain why collaboration through a cross-sectoral and multidisciplinary public private partnership is needed in particular:

Why does it require collaboration among several industry sectors (e.g. pharma, vaccines, biotech, medical devices, in vitro diagnostics, radiotherapy, medical imaging health ICT)?

Why does it require collaboration between private (industry) and public partners (e.g. academia, healthcare practitioners, patients, regulators)?

No medical technology developer alone can truly develop a medical technology specifically for such an unmet need and small, but collectively large numbers, as rare diseases. Currently, little is known about the evidence needed for an economic evaluation, neither from a public-private nor a public perspective. To truly make an economic framework for rare disease technologies, medical devices, in vitro diagnostics, radiotherapy, medical imaging, ICT and other industry sectors need to work together, as the products can be very diverse. But most importantly, given this topic, academic HTA researchers, HCPs, surgeons, industrial and academic developers, rare disease patients, regulators and HTA evaluators need to work together to set up the framework, as none of these can do it alone.

Applying the Medical Device Regulation 745/2017 has raised fears that some essential medical devices may be withdrawn from the market. Devices that treat rare diseases are particularly vulnerable as low-volume sales, and reduced return on investment characterise the market dynamics. As a result, a public-private partnership has excellent potential to protect devices at risk of being lost, in addition to supporting device introduction in future.

Furthermore, a report from the Boston Consulting Group in April 2022 noted that 89% of respondents now prefer market introduction in markets outside the EU due to improved predictability of requirements. As devices for the treatment of rare diseases are particularly vulnerable to market withdrawal, developing a predictable market and reimbursement pathway will help to ensure that essential devices are not lost.

Why is the contribution of industry needed to achieve the expected impacts?

***Contribution of industry:** Large companies that are members of the IHI industry partners (i.e. COCIR, EFPIA, EuropaBio, MedTech Europe, Vaccines Europe) contribute to the programme, primarily through 'in-kind' contributions (e.g. their researchers' time, laboratories, data, compounds). At least 45% of each project's total costs have to be in-kind contribution.*

While there are few orphan devices on the market, most medical technologies for rare diseases are developed within the industry. Quite some devices used for the treatment of rare diseases often include the 'off-label' use of devices authorized for other indications. As such, industry collaboration must support the identification of products, their way to market and materiovigilance. No company alone can develop a supportive harmonised for both regulation and HTA for this class of technologies.