

Topic idea submitted to IHI - Reference Number: TI_001199

Are you submitting the idea:

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

Please indicate the name of the group organisation: Inspire2Live

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

Patient/citizen organisation

1 Title of your idea

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

Failure Medicine data for further research - Building a consortium of patients, research institutes and industry

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:

In the databases of industry and EMA is a lot of data of medicines that did not get market approval. It appears for decades that this data is difficult to use. Either within the company itself and even harder between companies and research institutes. IHI can facilitate this with a consortium.

Medicines get market access when they meet with the 'Inferiority Principle'. This is EMA regulation. For example, when a medicine responds in 22% of the patients in a trial and an existing treatment responds in 23%, the medicine doesn't get market approval. When it works in 23% or more it does. For the patients in the 22% group this medicine, however, is very successful but not available. Under strict conditions the patients in the trial can stay on the drug for as long as it works for them.

Since personalized medicine becomes more and more available, the possibilities of determining what treatment might fit the best for an individual patient, these failure medicines become more interesting. This medicine might not only work in 22% of the patients in a specific breast cancer trial (example), it's also possible that it works in 12% of prostate cancer patients, 17% of pancreatic cancer patients and 32% of ovarian cancer patients. This medicine is a big success in the realm of personalized medicine, but still it's on the shelf of industry. By using the data, perform good research and hopefully bring the failure to a market medicine, we can do a better job for patients.

We should build a consortium of research institutes and industry that brings together, under strict conditions, the data of failure medicines for further research. Contributing institutes bring in the data and the money for research. This is paid by industry, for they will get the benefits when a failure drug gets market approval. Contributing and using the consortium is not for free.

Because we're dealing with patient data, patient advocates should participate in this consortium to

tackle, in cooperation, the right for sharing and the privacy issues.

There is another strong need for advocacy. Some industry partners might hesitate. Advocates can help to convince them and have them participating in the consortium. They show industry the need for sharing and research. They might even show them the possibility of bringing money, that was written off, back to life again.

This should be a pan European initiative with as much industry partners as possible and preferably as much research institutes also. There is a big role for EFPIA and IHI.

What might be a possibility, when a medicine is being known as successful and get market approval, is to organize an auction and have the highest bid win. The money flows back into the consortium. This idea for an auction has been launched a decade ago by Sage Bionetworks/Stephen Friend. It failed then but can be used now.

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

["SO1: contribute towards a better understanding of the determinants of health and priority disease areas"
"SO2: integrate fragmented health research and innovation efforts bringing together health industry sectors and other stakeholders, focusing on unmet public health needs, to enable the development of tools, data, platforms, technologies and processes for improved prediction, prevention, interception, diagnosis, treatment and management of diseases, meeting the needs of end-users"
"SO4: exploit the full potential of digitalisation and data exchange in health care"
"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:

["Non-communicable diseases"
"Cardiovascular diseases"
"Immune system diseases"
"Infectious diseases"
"Metabolic diseases"
"Neurodegenerative diseases"
"Oncology"
"Communicable diseases"
"Rare diseases"
"Paediatric"
"Prediction"
"Diagnosis"
"Treatment"
"Disease management"]

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

We should have the Pharmaceutical Industry bring in and make available their failure data for research in a data environment. Research both by industry and research institutes, in cooperation with patient advocates to determine the needs of the patient can benefit from this. We learn why medicines did not work in groups but sometimes in individuals. This will lead to knowledge that leads to personal treatments. Probably the failure medicines will lead to market approval because it will become clear which groups or individuals benefit from this.

There are still so many unmet medical needs in many diseases. There is a good chance that in the data answers for these needs are to be found. Unused patient data can now be used for the benefit of patients themselves and we can also meet with the interests of research institutes and industry. A win-win-situation can be created.

The objectives of this idea are to promote:

Can we find opportunities for failure medicines that benefit patients?

Can we structure the data in a way that it can be used for this research? FAIR might be an obligation and help in this.

We need modern techniques that can test the data for relevance and quality.

Can we target specific data to a specific population?

Start with a proof of concept or with a pilot (preferably) and kickstart when successful?

We need to bring different stakeholders together. Advocates, research and industry setup this consortium.

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.

Justification

The idea would fit in the IHI general and specific objectives, as referred to in the articles 116 and 117 of the Single Basic Act¹ and in the Strategic Research and Innovation Agenda. In particular, the idea:

Covers an unmet public health need, as defined in article 125(1) of the Single Basic Act;

I should say, definitely. So many unmet needs have an opportunity to be fulfilled with medicines that did not get a market access. There is now a good chance. There is a possibility for drug repurposing. There might also cross-disease use of medicines come out of this.

Is ambitious/innovative and goes beyond the state of the art;

This has never been tried and only a few times be discussed as far as I know. Until personalized medicine this was found too ambitious. Both EMA and FDA know that a lot of secrets are in the data of their organizations and the industry. Advocates can help to open this box.

Focuses on pre-competitive health research and innovation;

Definitely. The data of the industry is shared with all the participating partners. Pre-competitive.

Needs the IHI large-scale, cross-sectoral/multidisciplinary public-private partnership.

Without IHI this will not happen. Industry sees this as very competitive and IHI can bring in the pre-competitive phase. As said before; an auction can be the last phase of bringing a failure to market access. This is the moment competition starts.

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)?

It goes without saying that this only can be a success when government (EU/IHI) partners with industry. One could say that it also relates in a way to EHDS.

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.

The data is available at industry. And they can, as can the patient and research, benefit from it.