Report of the Internal consultation on patient engagement sustainability
(Expert Patient and Researcher EURORDIS Summer School 2018)

Summary

In June 2018, an internal consultation was organized by EURORDIS to explore with its members the different dimension of the sustainability of the patient engagement in medicines R&D. The consultation was conducted during the Expert Patient and Researcher EURORDIS Summer School (ExPRESS) face-to-face training held on 11-15 June 2018. EURORDIS co-leads the work package on sustainability in the IMI project PARADIGM and it was felt important to hold this consultation in order to help eliciting the position of EURORDIS and to be able to more accurately represent the views of its membership.

The consultation was preceded by a quiz in the morning in which all ExPRESS students had the opportunity to participate. The quiz consisted of 8 simple questions to test how students felt about their previous engagement experience, their priorities to improve it and how the future patient engagement framework in Europe should be organized in terms of governance and funding model.

The consultation was conducted in the afternoon and included two exercises. The first exercise consisted in asking the participants what makes patient engagement sustainable to trigger some discussions afterwards. The very open answers spanned from efficient training to open, trusted and coherent financial fluxes, in order to create value for all the players involved.

In the second exercise, the three pillars of the sustainability were discussed, namely processes, resources and culture. Within those pillars, the specific elements of governance, trust and revenue stream and financial viability were identified as the most important to support and ensure sustainability of the patient engagement in medicines R&D on the long term.

Altogether, this internal consultation was seen by EURORDIS as a first step of a series of activities (benchmarking, grey literature and one-to-one interview) to forge EURORDIS position.

Participant demographics

Following the launch of a call for expression of interest among the participants to the 2018 edition of the EURORDIS Summer School, 11 patient representatives were identified of which 9 attended the discussion. The discussion took place on 14 June 2018. Six different nationalities (Italian, French, German, Spanish, Bulgarian, British) were represented. Participants' ages ranged from 20-55 years of age. There were 8 females and 1 male.

Moderators

Mathieu Boudes (European Patients Forum)
Elisa Ferrer Mallol (EURORDIS)

Context and background

Patient engagement medicines lifecycle is a trendy topic. Although it is the daily life of patients and patient organisations, it has become over the past years, a hot topic for the community of all actors involved in the medicine lifecycle. However, in practice its application varies greatly across stakeholders, countries and disease areas. Indeed, patient-centeredness is often approached superficially, focusing on patients'/citizens' choice and satisfaction rather than seeking patient's input into design. Moreover, there is a common acknowledgement that the engagement is fragmented, inconsistent and often far from being optimal, as a result of years of isolated engagement practices with good (and bad) examples.

In other words, patient engagement is hot but in its adolescence but clearly it is a field in progress even though good patient engagement practices are not yet firmly established across the stakeholders and along the value chain.

Achieving a structured and systematic patient engagement in a sustainable and impactful way requires acting on system readiness, building capabilities for engagement among all stakeholders, based on three pillars: (i) patient education and training, (ii) development of non-competitive tools for engaging and (iii) sustainability of patient engagement in terms of concept, resources and processes. Only when all three aspects are covered can patient engagement become the norm.

One of the greatest challenges to be addressed in the coming months is the sustainability of patient engagement processes that will encompass a 360° strategy, to leave no one behind, no one frustrated and everyone engaged because patient engagement is everyone's business.

Clearly, there is a need for all stakeholders involved in the medicine lifecycle to have a structured, open and frank dialogue about a sustainability framework, without any taboo. This would already represent a meaningful step followed by the development of a comprehensive sustainability strategy. The next milestone will be the development of an operational roadmap including the catalogue of services that will fulfil the expectations and needs of all future users and/or clients (e.g. companies, CROs, academic research teams, regulators, HTA), the definition of an operational framework that is suitable for different user groups, and governance and business models that would sustain the necessary infrastructure[1].

Summary of the discussion

Exercise: phase 1

Participants write down their answers to the question *What makes patient engagement sustainable?* and afterwards everyone shares them with the group

Responses to *What makes patient engagement sustainable?*

1 – More **training**, **reimbursement and travel expenses covered**, carers to be allowed to participate, (more webinars as opposed to face-to-face), providing translators, forums and online help for questions and having an online form to collect feedback.

[1] Patient engagement in the lifecycle of medicinal products: Towards a sustainable implementation Boudes M, Hivert V, Ferrer E, Le Cam Y and Andersen T (p12)
https://www.diaglobal.org/_GlobalForum/2017/Nov2017/index.html#

2 – Better **funding**, not only from governments but also a mandatory percentage of drug developments costs, as pharma needs to ensure budget for patient engagement; even to penalize companies and academia if they are not budgeting for patient engagement (PE); it has to be a top down implementation, there is no PE in a self-regulated pharma world. More natural history studies, we have to ask patients for their needs and symptoms (scary that pharma knows so little) and also educating healthcare professionals about value of patient engagement using concrete examples.

3 – I was never encouraged to become an educated patient representative by anyone in the healthcare system. There is a gap that should be filled with information to the patient by the physician encouraged, because one cannot become a patient representative and provide sustainability in patient engagement if you don't have patients that know that they can do it.

4 – When becoming engaged there should be a system of compensation in place, reimbursement of expenses, but also making it easy for patients to engage: consider the location, time of the day for conference calls, using skype. Patients will become more motivated to engage if they know that their contributions are valued and actually implemented, regular feedback on contributions should be provided (agreement in the room). As a patient you also need to know that you are making a difference for others

5 – To make patient engagement sustainable, I need to be motivated and to be acknowledged and valued, if these factors are present the patient feels motivated and empowered to be actively engaged even if financial resources are not there. Also to have the feeling of belonging to group with a defined purpose. Financial resources should mainly be addressed to participate in events, conferences and training opportunities. It is important to feel harmonized in a system e.g the system of Rare Diseases. For example, here in the Summer School we are all at the same level, patients, regulators. We need to be equal partners.

6 – Motivation is very important and feel appreciated for your work. At the same time, financial resources are also important (reimbursement and compensation framework). Creating a network and use social media more often and actively, as communication is very important. Also to establish training programmers to be organized more often, to have the opportunity to meet fellow patients and share knowledge and create better things for the future.

7 – EU kind of funding, limited eg 5 years, does not ensure sustainability. For example, successful networks created in one project are not part of the next framework, it is a waste of resources. Patients can be really motivated [within a project], but in 5 years it is over.

9- I wanted to think about the experience I've been through 10 years as part of European patients' organization and about the hurdles we've been facing along the way. And also I wanted to envision what would be ideal to have in the future: money to support the good functioning of patient associations; training to form new generations of expert patients, not just training of patients but also healthcare professionals, researchers, i.e the key stakeholders (I've seen many times that healthcare professionals can dominate the group of patients, and this domination has to come to an end); also to access best practices with a special focus on new technologies, best practices in fundraising, on how to engage with key stakeholders, on better ways of running a patient organization; avoidance of unnecessary workload; break down language barriers and foster better communication; to be up to date with legislation, norms; and legal support. Incubators – really like to be involved as a patient

10 - Motivation as in any job, even this is considered a job even if not paid, also value and the feel of belonging to a group (particularly important in the rare disease community), there is a lack of communication, many associations don't know about training and they don't care, they think they are protected by their clinicians and researchers. And although most of them care, they also have interests, they want to publish their work. Patients should be leaders about what they want to do. Researchers and clinicians don't know anything about the regulatory and when they want to push a molecule to licensing, the data that they have gathered might not be what is needed from the regulatory point of view. There is lack of communication of regulatory world and researchers. Patient associations need to invest in lawyers, regulators group associations need to have access to specialists in this area. Think long term even if there is no success this will help future therapies, in the moment that you give data to big pharma you are in a way selling yourself, think about what you want, not the other way around, patients need people that know how to help them.

Moderator: What is the important thing(s) that were said? Prioritisation

- To get rewards and recognition of what you have done
- Training and education of patients to be able to engage with the different stakeholder groups. To enhance the capacities of patient organisations.
- Training and education of researchers on how to engage with patients
- Regarding the lack of communication: Patients and clinicians (for example) overlap only when there is an ongoing clinical trial. But when the trial stops, communication is not at the same level as previously. There should be continuous channels of communications so everyone is at the same level of information. From the patient organisations' side there may be a need for support to know what kind of research is ongoing in pharma and academia. Alignment of all stakeholders all along the cycle of the medicine and reporting from both sides. Patient organizations don't have capacity or knowledge to do that. Example of the lack of communication: after a failed trial pharma support disappeared. But since the trial was still ongoing at the NIH, we (patient org) created a consortium of all researchers, and established that researchers should meet face to face once a year, to bring them closer. Comment: this needs resources, for academia it's a job and academia might not have the resources to do that. Better funding mechanisms may be needed.
- Training without engagement does not have a point. And engagement without training is not effective.
- Creating effective platforms for registries by patient organisations. Data of these registries will be owned by the patients and this is a fundamental asset. But this might create competition between patient organisations (how do we keep our market). We are moving away from the registry structure, which is also hosted by healthcare professionals most of the time. The use of technology may prevent this kind of competition and will be used to redistribute the value to where it came from.

Exercise Phase 2: Discussion of the sustainability dimensions

Moderator introduces the model of the three pillars of sustainability:

- Culture: changes needed to make patient engagement the norm; system readiness)

- Processes: driving stakeholders together (trust)
- Resources (economic resources towards a non-profit objective)

And present the results of one of the questions from the morning session:

- Q5: Who should organise it (the engagement of patients)
  - A partnership between the EFPIA and the EMA: 5 votes
  - An independent NGO, as a foundation from a patient organization: 2 votes
  - Disease-specific patient organisations (European Federations): 10 votes
  - Umbrella patient organisations (EPF or EURORDIS): 6 votes
  - A body from a EU public institution: 2 votes
  - As it is right now, it works: 0 votes
  - Something else: 0 votes

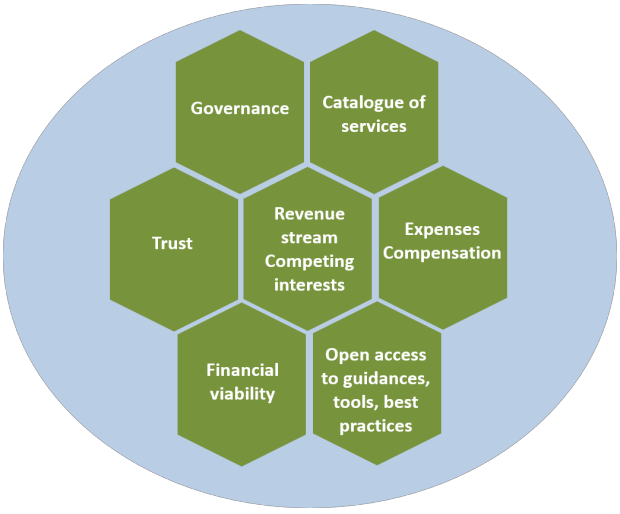
This question was provocative in purpose and aimed at stirring the discussion about potential models and potential actors to organize patient engagement in medicines R&D.

Moderator stated that an expected response was that patient organisations should be highly involved, but a surprising result was that some patients voted between a partnership between the pharma corporate syndicate and EMA.

Comments from participants

1. There should be a partnership between 3 partners: an European body or EMA, pharma and patient organisations
2. Mixture of EFPIA and EMA is the right one
3. Patient engagement funded by EFPIA and EMA but independently organized by patients
4. Only organized by patient organizations but in a rotating scheme, like EU presidency
5. The question wasn't well formulated
6. We need something new and better, who should have ownership
7. Participant trusts EMA for funding, trust the syndicate less, good combination of the two

Moderator showed that any potential sustainability model should consider the following dimensions which are needed to systematize meaningful patient engagement.



Moderator: what dimensions are the most important? The group agreed the following prioritization:

1st. Governance
2nd Trust
3rd Revenue stream and financial viability

Brainstorming around these three dimensions:

Governance

Moderator: who should organize it? Who should govern it?

1. European consortium, public private partnership (PPP)
2. New creation, new public private partnership

Moderator: PPP may be slow in the decision-making, is this the only model can work?

3. Another option: public-only consortium: partnerships between national agencies, families, ministry of health, social
4. Not only European national body, but also in collaboration with patients, preferably an umbrella patient organization not to push for a particular disease. To avoid being only politics-driven. Patients should be controlling. Double governance.
5. Include groups of researchers, also carrying out EU projects, learned societies. They could be part of this consortium.

Moderator: How do we organize the system – who should be the boss?

6. Patients should be the boss - European umbrella patient organization to avoid disease prioritisation
7. Need to avoid that the boss is always is the same, to give the opportunity to groups that were not involved before
8. Creating a new bureaucracy and new organisation, it has to be professional. And we need to have the funds for that.
9. We need extremely qualified people/patients, ensure that we always have qualified patients
10. Creating a new generation of patients who work in favor of overall community of patients. This should be set in stone in the governance.

Moderator: Professionalization of patient organisations, keeping the value of the volunteer work but with professional processes

Trust

Moderator: How do we build trust and mitigate distrust?

1. Write a charter
2. To be very transparent because you are in the spotlight

Moderator: Do you see trust as an outcome of professionalization or you build on?

3. It's not just technical, it is soft skills its values, passion, motivation, leading by example

Moderator: transparency alone doesn't solve it – people do bad things very transparently

4. If you become too professional (as a patient organization) this takes away trust, people don't understand that people who are not affected by the disease suddenly are taking decisions on behalf of patients. One example: loss of trust in the patient organization after moving offices to a trendy location – the perception by other stakeholders is important.
5. Concrete robust work – to lead by example

Moderator: If you start from scratch which are the elements to create trust?

1. Good PR
2. Open communication
3. Time and agreement on common goals from both parties (long-term)
4. Good examples
5. Written charter, the trust builds on that
6. Allies, having allies outside the rare disease space
7. Example of one organization: Researchers are creating opportunities for patients, patient associations creating opportunities for them but telling patients about their work, recruit patients. Mutual benefit.
8. Checkpoints to make sure that your entrusted people are the right ones

Moderator: This mean building solid processes that will help towards the culture change and to enhance the trust

Revenue stream and financial viability

Moderator: Money is in pharmaceutical companies to carry out drug development. Regulators/HTA and academia cannot pay. The only funders are the pharma and then there is an exchange of money from pharma to something else managed by the European umbrella. A business model in patient engagement would be not for profit is just to be ensure that the activities can be performed. A potential model could be fee-for-service: pharma pays for a certain type of patient engagement (catalogue of services) with a defined price. Another potential model could be a subscription model in which Pharma A and B both put money in a common pot that is managed by this other entity with a multi-stakeholder governance. What are your opinions on that? Do you think they could be possible? Do you see another way?

1. Exclude fee for service – Pharma then they have control, the fee may not mean much for them. Membership model: it aligns with the model used by many patient organisations. Being a member means "I am part of this".

Mod: why the fee for service should be excluded?

2. When there is no profit there shouldn't be an exchange of money.

3. But the fee for service does not necessarily mean that there will be profits (fee used to cover operating costs).
4. Would prefer first option, i.e. fee for service. A flat fee does not seem to me really fair, because you may have completely different overheads depending on where your organization is based.
5. Services may be different depending on the needs, and therefore fee should be different. If every company, big or small, is putting the same amount of money (subscription model), the big company would be getting the services almost for free.
6. In our organization we operate with a similar model. For example, a Facebook post costs 250, but we tailor the fee or even waive it if the company is a small start-up with projects aligned to ours.
7. I still have problems picturing this model because it may lead to competition between volunteers/patients inside a patient organization. How much is my engagement worth?
8. But patients are not being paid in this model. The pharma is paying for a service, not directly to the patients, but to the institution with this governance. The patients are not employees of this institution, there is not profit made.
9. I still see the risk of this thinking about worth even if no money exchanged
10. There are cultural differences when talking about money and it is true that this may be differently perceived in the US vs Europe.
11. How would you feel about something similar to corporate social responsibility, big company gives more? And smaller companies give less. A company would give a percentage of their revenue to this umbrella organization who would distribute it.
12. What if general consumer companies, other than pharma that would not have direct interests, could give money to this model.

Moderator: This model may need a bigger cultural change. Going to general public is another step.

13. I think it would be a way to make patient engagement more public. The general public would realize about the big problems related to medicines development. Open it to the public

Moderator: That could be a model based on corporate social responsibility open to stakeholders outside the medicines development

Moderator: I would like to challenge corporate social responsibility. Is it sustainable?

14. That would be the way to calculate a percentage, to define an amount. It goes along with the same discussion about medicines prices and making the healthcare system sustainable. Patients generate value, company gets a price, someone has to pay, but who was instrumental in the development of the medicine? The patients and they need to be rewarded, and that is how you calculate this percentage on the sales. But for this to be sustainable it needs to be written in the law. But for this need we need to define the patient engagement's value.

Moderator: This may work in the long term, but not in the short term.

15. Subscription will not work because disease-specific patient organisations would want to renegotiate with the pharma, creating a competition between middle man (umbrella

organization) and disease specific organizations. If everyone would like to re-negotiate the system would collapse.

Moderator: But if we go with the fee for service, we charge 100, 80 would go to operations and 20 of profit. Those 20 they could be a mechanism to refinance disease specific organization, that would be a way to cascade the money and to build capacities of organizations that are engaging or wanting to engage.

No other comments provided. Session finalizes.