



RECAP preterm

Collaborative Research on Very Preterm Birth: Concepts and Methods – Working with the RECAP preterm Data Platform

Dissemination, Translation and Sustainability

Juliëtte Kamphuis **The value of patient representatives**

From a patient perspective: The importance of patient representatives as partners in research projects



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Hi, my name is Juliëtte Kamphuis, from the Netherlands, and I will give you some insight into the value of patient representatives as partners



You were born preterm.
Would you like to describe for us
your personal story?



Question: You were born preterm. Would you describe for us your personal story?

Too curious for the world!



- Born very preterm at 28.5 weeks in 1980
- Mechanically ventilated and severe bronchopulmonary dysplasia as a baby
- Mis-diagnosed from age 26-40 with difficult to treat asthma (2006-2020)
- Re-diagnosed with bronchopulmonary dysplasia age 40 (2020)



1980: 28.5 weeks, 1250 gram – 37 cm



1981: 1 year old



Current



Too curious for the world I was born very preterm with 28 weeks as you can see in the first picture.

I was mechanically ventilated as a baby and my lungs always remained the weakest point growing up. As a young adult, my lung issues became more severe and this influenced my daily quality of life.

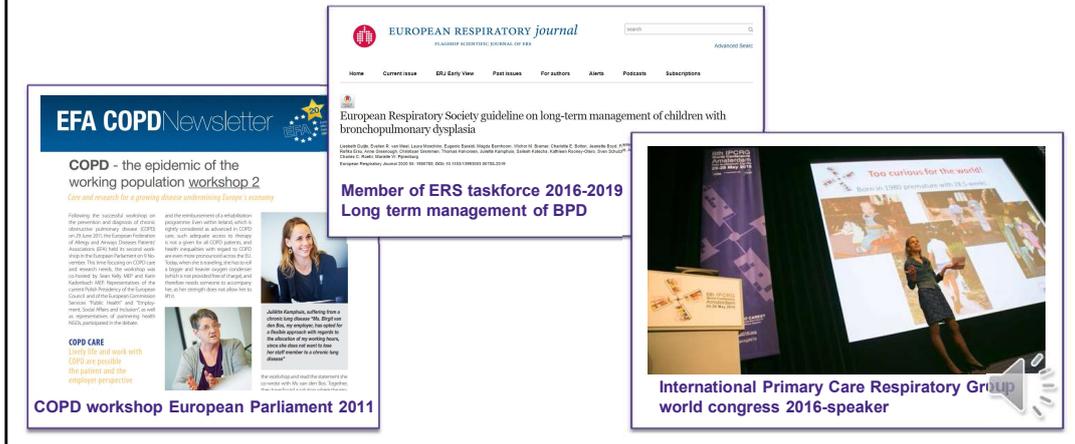
I was mis-diagnosed with asthma for a long time and I received the right diagnosis of having BPD in 2020. Finally now, I also receive the right care.

Even though I have a lung disease, I still enjoy being physical active and I love running, cycling and hiking as you can see in the other picture. I have to work hard to remain a good condition and it is also the process I love, puzzling to achieve certain goals when in my case, my lungs are not very nicely developed missing alveoli throughout my lungs and the lowest part of my lungs are more injured.

Using experience to improve healthcare



- Patient representative since 2009
- Raising awareness about lung diseases and preterm birth
- Collaborating with national, European and worldwide organisations
- Involved in research projects and healthcare related initiatives



Coming from a negative experience as having a lung disease, I use this for the better, namely to improve healthcare. Since 2009 I am a patient representative beside my daily work. I raise awareness about lung diseases and preterm birth. I collaborate with national, European and worldwide organisation. And I am involved research projects and healthcare related initiatives.

These pictures show a few examples I did in the past.

The picture on the left shows me during a COPD workshop held in the European Parliament, which was focusing on COPD care and research needs. During this workshop I read a statement which I co-wrote with my former employer and addressed how we found solutions and which approach is needed from an employer to keep me active in the working environment while having a chronic lung disease.

In the middle it shows a guideline on long-term management of children with BPD, which was published last year in 2020 Together with a parent of a preterm child I took part in this ERS Task Force and we provided our advice on which questions and outcomes were relevant for monitoring and treatment of children with BPD.

On the right you see me doing the opening of the world congress of the IPCRG and I was speaking about the ups-and-downs of difficult to treat asthma and discussed these matters with the audience.



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Why do you consider the inclusion
of patient representatives in
research projects important?



Why do you consider the inclusion of patient representatives in research projects important?

The value of patient representatives



- Knowledge of living with an illness
- Identifying research questions and relevant research outcomes
- Involvement in the design of the project leads to efficiency
- Dissemination of findings and act as ambassador

RESEARCH ARTICLE Open Access

Patient engagement in research: a systematic review

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Abstract

Background: A compelling ethical rationale supports patient engagement in healthcare research. It is also assumed the patient engagement will lead to research findings that are more pertinent to patients' concerns and desires. However, it is unclear how to best conduct this process. In this systematic review we aimed to assess a key question: what are the best ways to identify patient representatives? How to engage them in developing and conducting research? What are the observed benefits of patient engagement? What are the barriers and facilitators of patient engagement?

Methods: We searched MEDLINE, EMBASE, PsycInfo, Cochrane, ERIC, CINAHL, SCOPUS, Web of Science, Business Source Premier, Academic Search Premier and Google Scholar. Included studies were published in English of any size or design that described engaging patients in their caregivers or research design. We conducted an observational case of the study. We analyzed and synthesized with experts and patients. Six were analyzed using a conceptual framework. We used a template analysis.

Results: We included 10 studies that described a spectrum of engagement. In general, engagement was feasible in both single and multi-center sites. The barriers of health inequity, setting and resource development, and low community during the selection and retention of research. We found no comparative impact study to implement a patient research. Patient engagement increased study recruitment rate and patient research in security, family, caregiver, study process and, sharing relevant outcomes. The most commonly cited challenges were related to patient representation and the time and funding needed for engagement and conducting study of a systematic engagement.

Conclusions: Patient engagement in healthcare research is likely feasible in many settings. However, this engagement comes at a cost and can become burdensome. Research indicates to identify the best methods to achieve engagement in a timely and clearly needed.

Keywords: Systematic review, Patient Engagement, Patient centered outcome research

RESEARCH CASE & HEALTH

Patient Engagement: Four Case Studies That Highlight The Potential For Improved Health Outcomes And Reduced Costs

By Anne L. Lawrence, Sara S. Henderson, Peter J. Havelit, Melvin Meier, Hester H. Kavan, and Organization, and the Best

Abstract

Background: While patient groups, regulators, and sponsors are increasingly considering engaging with patients in the design and conduct of clinical development programs, sponsors are often reluctant to go beyond pilot programs because of uncertainty in the return on investment. We developed an approach to estimate the financial value of patient engagement. **Methods:** Expected net present value (ENPV) is a common technique that integrates the key business drivers of cost, time, revenue, and risk into a monetary metric for comparing projects. We used the ENPV to compare the expected net present value of a patient engagement program versus a traditional program. **Results:** For a typical program, the cumulative impact of patient engagement over the course of a program can be substantial. For example, a program with a 10% reduction in time to market, a 10% reduction in cost, and a 10% increase in revenue can result in an ENPV of \$100 million. **Conclusions:** Risk-adjusted financial models can assess the impact of patient engagement. A combination of empirical data and subjective parameter estimates shows that engagement activities with the potential to avoid preclinical assessments and/or improve enrollment, adherence, and retention may add considerable financial value. This approach can help sponsors assess patient engagement investment decisions.

Research Engagement

Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project

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Abstract

Background: While patient groups, regulators, and sponsors are increasingly considering engaging with patients in the design and conduct of clinical development programs, sponsors are often reluctant to go beyond pilot programs because of uncertainty in the return on investment. We developed an approach to estimate the financial value of patient engagement. **Methods:** Expected net present value (ENPV) is a common technique that integrates the key business drivers of cost, time, revenue, and risk into a monetary metric for comparing projects. We used the ENPV to compare the expected net present value of a patient engagement program versus a traditional program. **Results:** For a typical program, the cumulative impact of patient engagement over the course of a program can be substantial. For example, a program with a 10% reduction in time to market, a 10% reduction in cost, and a 10% increase in revenue can result in an ENPV of \$100 million. **Conclusions:** Risk-adjusted financial models can assess the impact of patient engagement. A combination of empirical data and subjective parameter estimates shows that engagement activities with the potential to avoid preclinical assessments and/or improve enrollment, adherence, and retention may add considerable financial value. This approach can help sponsors assess patient engagement investment decisions.

Outcomes from the other side

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Abstract

Parents and individuals who were born preterm rarely contribute to research study design in order to ensure that outcomes are reported that are of relevance to them. In this article we explore aspects of the measures we use and the lived experiences of these individuals with experience of having a very preterm birth or being very preterm themselves. Their experiences tell us that follow-up needs to be more than 20 years, that perinatal early to more widely acknowledged in education and that adult services need to consider the consequences of being born early. There are encouraging signs that these important issues are becoming recognized. Individuals designing outcome studies should ensure that these important voices are heard, and their perspectives captured in such studies.

(Please note: these publications are meant as examples)

Knowledge of living with an illness

Patient representatives, such as parents and carers of preterm born children, and former preterm born individuals, have a different kind of knowledge, namely as a consumer of the health care system and knowledge about living with an illness or a condition due to preterm birth.

Identifying research questions and relevant research outcomes

I experienced myself as being a patient representative that this so called "lived-experience" is crucial and very helpful for identifying the right questions to ask and which outcomes are relevant to measure and making sure that the project has relevance.

Involvement in the design of the project lead to efficiency

There is also several suggestive literature out there that involving patient representatives into the design of a research project may lead to a more effective study and perhaps may lead to cost efficiency as well. It is not only my personal belief!

Dissemination findings and act as ambassador

Patient representatives could help to disseminate findings and act as ambassador. They can help to explain why certain aspects of the project are so important, plus to disseminate the research results back to the society.

I highly recommend to use the patient voice, as I already addressed previously in the article published in Seminars in Fetal and Neonatal Medicine last year;

Invite parents and carers to the research table, invite also former preterm young adults and even children.

Combined they have a very strong voice politically and, If we want to fully understand the impact of preterm birth and what is needed in healthcare for preterm born infants growing up, we should include their perspective; individuals born preterm plus parents and/or carers.



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What are currently the biggest challenges regarding the inclusion of the patient voice in research projects for you?



What are currently the biggest challenges regarding the inclusion of the patient voice in research projects for you?

Challenges can be fun!



- Involve patient organisations early
- Involve parents/carers of preterm infants & preterm born individuals
- Facilitate, support, coordinate and communicate
- Provide patient representatives with training
- Budget for patient involvement: don't forget!



Involve patient organisations early

Involve patient organisations as project partners as early as possible, before grant submission. And of course not 2 days before grant submission to receive their checkmark. It should be done in a way they have sufficient time to provide their feedback about the proposal. Be clear about their role also in the project as a partner

Involve parents/carers of preterm infants and former preterm individuals

Ask patient organisations for help to find parents or carers of preterm infants and former preterm individuals, young adults and children in order to install a patient advisory board. Think of the condition or issue that they must have experience of, or from a range of countries, and socio-economic groups that should be covered. For example, a vacancy could be created with the help of a patient organisation and this could be spread via social media.

English is often the main language in research. This could be a potential barrier to find persons from a range of countries where English is not their native language.

Be creative in finding solutions for this and work for example together with local patient organisations, they could for example help to install a local focus-group, and could offer their help with translation of text into their native language, if you experience difficulties in including the patient voice from certain countries.

Facilitate, support, coordinate and communicate

Patient organisations can help to plan, guide and coordinate the patient involvement during the project, or appoint a project member who can fulfil this role. Inform the patients representatives well during the project by having online meetings or face-to-face. Facilitate patient representatives taking part in conferences and events to showcase their involvement and the work of the project.

Provide patient representatives training

Make sure that patients representatives have the skills and knowledge needed to represent themselves and the wider community and that they have a basic understanding of clinical trials or research projects. For example EUPATI and EURORDIS have developed education for patient representatives.

Budget for patient involvement

Include cost for patient involvement in the project proposal budget

Such as cost of patient participation in annual meetings and events, budget for travel costs and accommodation. Reimburse patients representatives for their time, as often they need to take time off of their work.



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In your opinion, what are the key aspects to enable meaningful inclusion of the patient voice in research projects?

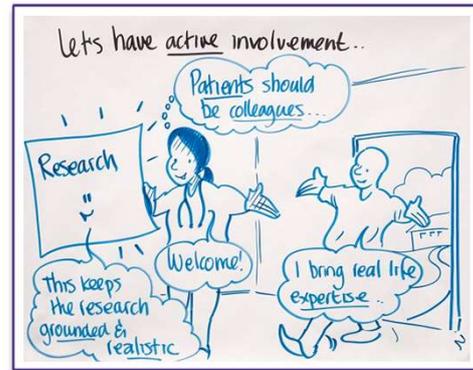


In your opinion, what would be the key aspects to enable meaningful inclusion of the patient voice in research projects?

Key principles of meaningful involvement



- Involve early, fully and meaningfully
- Promote and disseminate the outcomes
- Equal partnership and recognition



Key principles:

Involve early, deeply and meaningful

Involve the patient voice early in the design process and involve deeply in a meaningful way. The more patients representatives are involved in the daily activities of the project, and informed and updated about the progress, the more they can understand, contribute and have a positive impact.

Promote and disseminate the outcomes

Promoting the research project and disseminating the outcomes, goes much further than telling a personal story, however this can be very powerful and provides inspiration. Include the patient representatives for example to adapt scientific language for a non-scientific audience.

Equal partnership and recognising

Value patient representatives as equal partners. They spend their time, use their knowledge and expertise to make the project successful. If other project members are paid for their time, reward patient representatives too by being paid for their work. Value their work through news and articles on the project website and in scientific literature



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Could you describe a good practice example that you experienced as a patient representative?



Could you describe a good practice example that you experienced as a patient presentative?

Best practice example: U-BIOPRED



U-BIOPRED was one of the first Innovative Medicines Initiative projects (IMI) funded for a total of almost €27 million (run from 2009-2015)

U-BIOPRED consortium:
representatives of all stakeholders in 11 EU countries

20 academic institutions
11 biopharma industry partners (EFPIA)
6 patient organisations
3 small to medium enterprises
1 multinational industry



U-BIOPRED (Unbiased BIOMarkers in PREDICTION of respiratory disease outcomes) is a research project using information and samples from adults and children to learn more about different types of asthma to ensure better diagnosis and treatment for each person.

Patient Input Platform

- 11 patients from 5 EU countries
- involved in all stages of the project
- Active in:
 - Ethics Board, Safety Monitoring Board and Scientific Advisory Board
- A short guide and article shares the tips and best practices



I was involved in the past in U-BIOPRED, a severe asthma project, which was funded by IMI that clearly identified patient representatives as important stakeholders.

Patient organisations were involved early as partners even before the proposed project submission. They helped to recruit patient members and to set up a patient input platform and supported the patient members in all activities during the project

11 Patient members from 5 EU countries were active in the Ethics Board, Safety Monitoring Board and the Scientific Advisory Board, and were involved in all stages of the project.

For example, we helped in making the study design and consent forms more study participant friendly, for example by reducing the amount of hospital visits, adapting and shorten the study questionnaires, re-writing informed consent forms in lay language, which helped with recruitment and preventing drop-out of study participants.

We have shared in a short guide some tips on how to involve patients in research, and published also an article to share the best practices in patient involvement in U-BIOPRED. These are very helpful for successful patient involvement.



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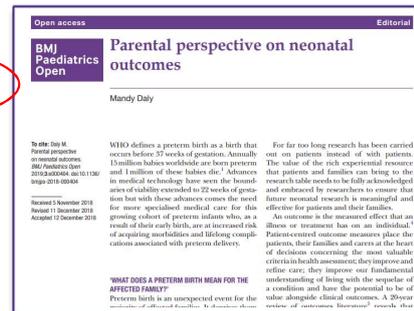
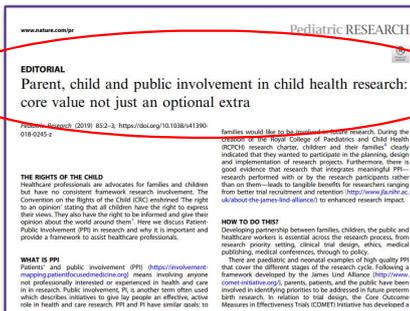
What would be your recommendations
for future research projects?



What would be your recommendations for future research projects?

Recommendations

- Change in mindset, be open-minded
- Educate yourself in patient involvement
- Use experiences and lessons learnt from others
- Exchange and discuss with patient organisations



Change in mindset, be open-minded

Including the patient voice well in research projects requires a change in mind set and to be open minded. Scientist sometimes may think “science is for scientist”, so how can a patient voice contribute to make the research project better and successful?

There should be a willingness to collaborate and to be convinced of the potential benefits of patient participation.

Educate yourself in patient involvement

There are a lot of e-learning modules online, workshops and websites to educate yourself about patient involvement. Contact patient organisations and ask if they could provide information for the best education to follow. I myself could recommend to follow the workshops from Conect4Children, to learn on how to involve family and children as partners into clinical trials, and I have also seen online that Paradigm has developed a nice toolbox to look into of which could be helpful.

Use experiences and lessons learnt from others

Use the experiences from other researchers on how they involved patients as partners in their research projects, and also if they have tips and how to make your research project the next best practice example.

Exchange and discuss with patient organisations

Exchange and discuss with patient organisations about patient involvement, and how to reach and represent those voices that are “seldom heard”. Think about how to make it possible to include more and more the persons who are living with the conditions of a preterm birth, or for example other childhood diseases. Also children should be asked to give their voice to research projects.

And remember; parent, child and public involvement in health research is a core value and not just an optional extra!

Thank you

In case of questions, please feel free to contact
research@efcni.org



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 733280.



Thank you

Thank you very much for listening to this module.

Please don't hesitate to contact me for questions, and I would love to help you with sharing my experience and expertise on how to involve parents or carers of preterm infants and preterm born individuals successfully into your research project as partners