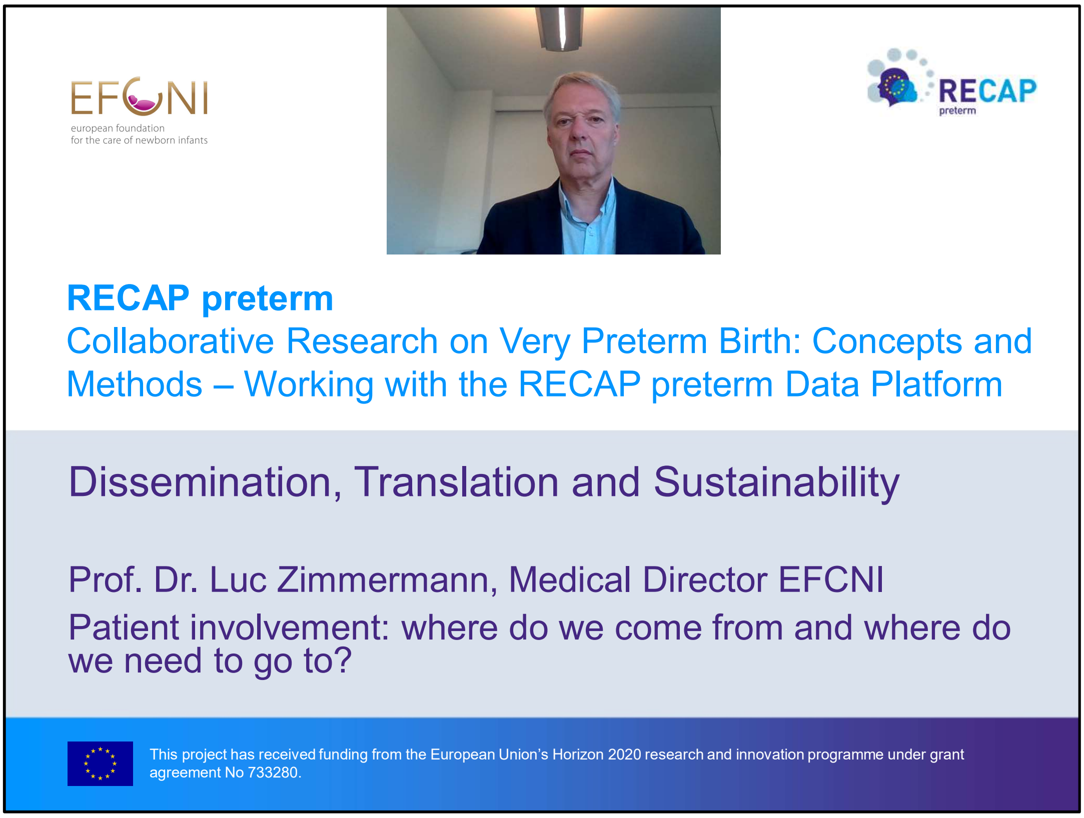

RECAP preterm

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

Dissemination, Translation and Sustainability

Prof. Dr. Luc Zimmermann, Medical Director EFCNI

Patient involvement: where do we come from and where do we need to go to?

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

Dear fellow researchers, ladies and gentlemen. I am Luc Zimmermann, Medical Director of the European Foundation for the Care of Newborn Infants, EFCNI. I worked many years as a clinical neonatologist and I am involved in research as a Professor at Maastricht University Medical Centre in the Netherlands. I will give an introduction to our topic "Involving families as partners in research projects" and will discuss where we come from and where we need to go to.

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Traditional patient role: receiving services

Information

Consultation

Prescription

Treatment/ Therapy

My doctor doesn't have enough time for me

My doctor decides what is best for me

My health insurance doesn't cover the therapy I would need

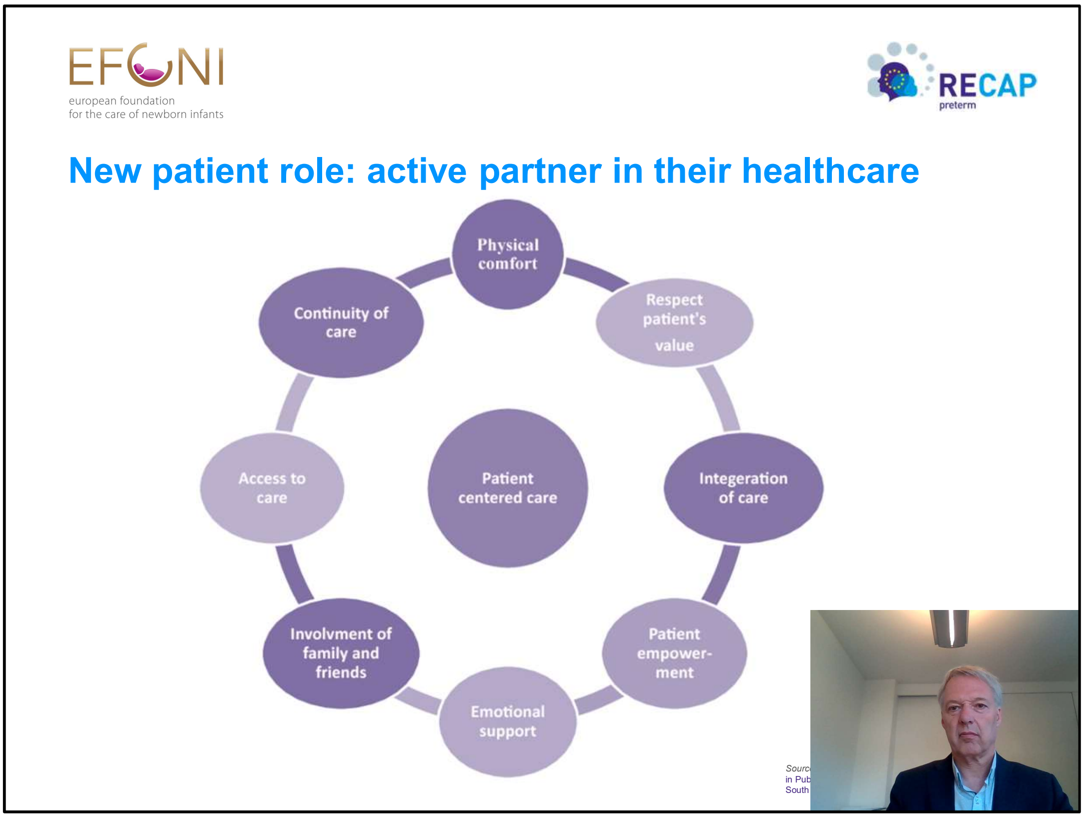
No therapy really helped me

I don't understand the effect of the medication

Who is taking care of me?

Adapted from: EFGCP, Dr Ingrid Klingmann

Traditionally, medical services were the main point of focus and delivered one directional. However, the real concerns of patients were frequently not solved. This quote is a good summary: „My doctor decides what is best for me“.



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New patient role: active partner in their healthcare

Patient centered care

Physical comfort

Respect patient's value

Integeration of care

Patient empower-ment

Emotional support

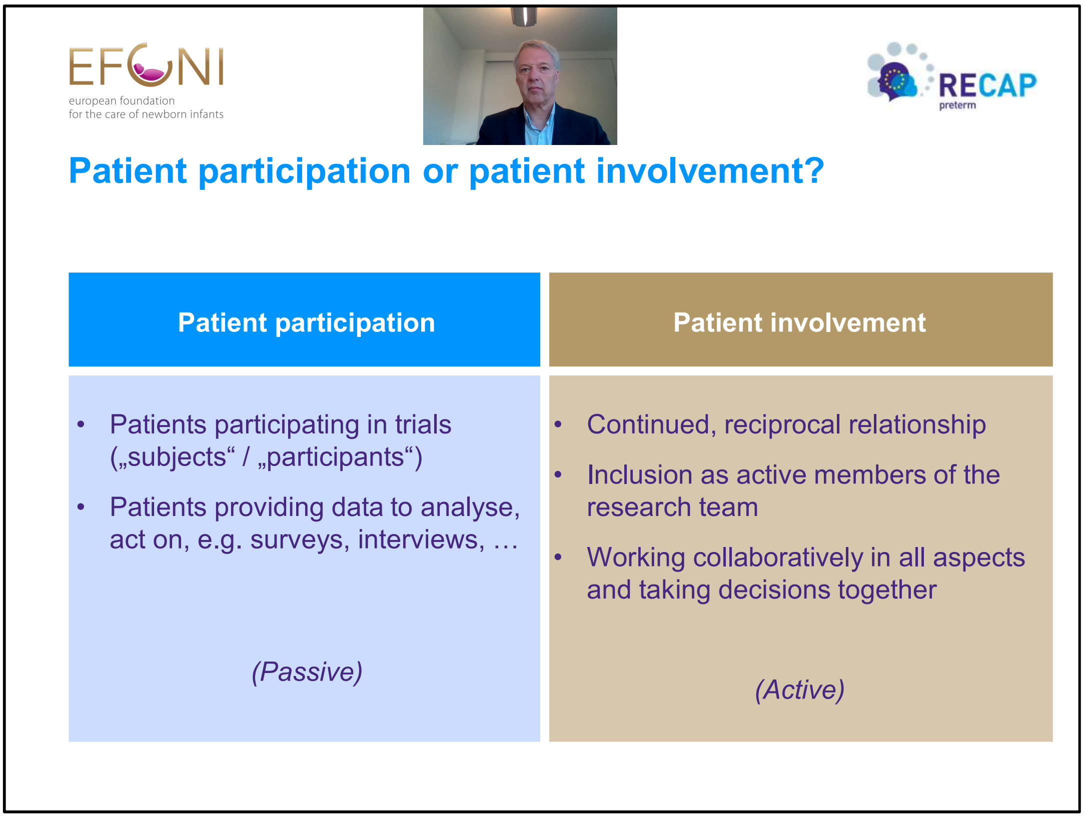
Involvment of family and friends

Access to care

Continuity of care

Sourc
in Pub
South

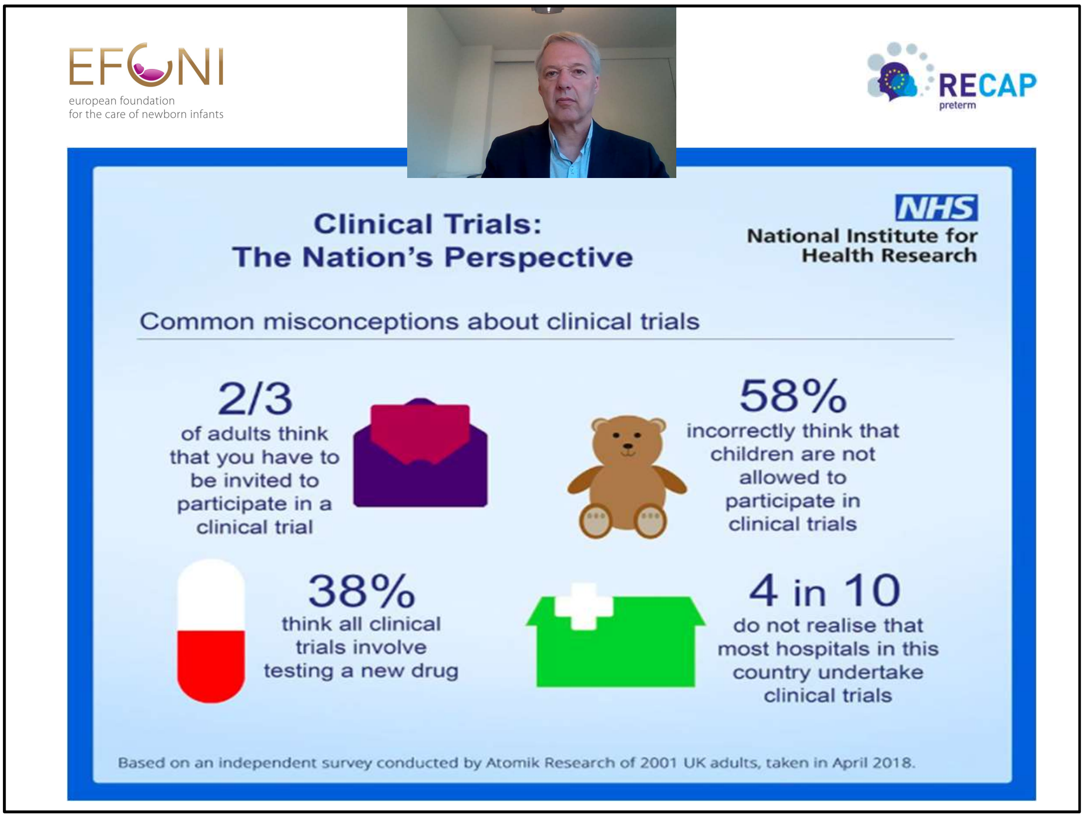
In the last years, patients have become an active partner in their own healthcare leading to the concept of „patient centered care“. This includes many aspects such as patient empowerment, emotional support and involvement of the family.



Patient participation or patient involvement?

Patient participation	Patient involvement
Patients participating in trials („subjects“ / „participants“) Patients providing data to analyse, act on, e.g. surveys, interviews, … *(Passive)*	Continued, reciprocal relationship Inclusion as active members of the research team Working collaboratively in all aspects and taking decisions together *(Active)*

Not just in healthcare but also in research the role of patients has changed and is changing further. We have to make a distinction between patients who participate in trials as subjects and the involvement of patients in research as an active collaborative member of the research team.



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NHS
National Institute for
Health Research

Clinical Trials:
The Nation's Perspective

Common misconceptions about clinical trials

2/3
of adults think that you have to be invited to participate in a clinical trial

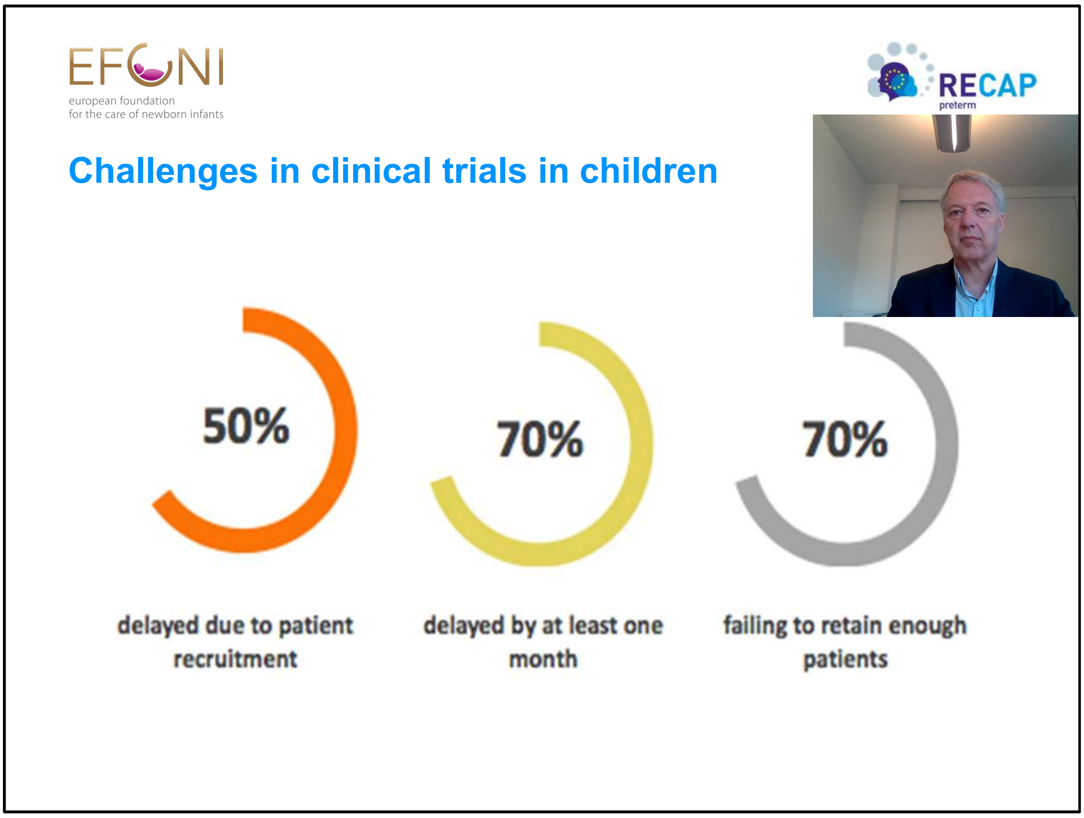
58%
incorrectly think that children are not allowed to participate in clinical trials

38%
think all clinical trials involve testing a new drug

4 in 10
do not realise that most hospitals in this country undertake clinical trials

Based on an independent survey conducted by Atomik Research of 2001 UK adults, taken in April 2018.

Patients and many stakeholders have many misconceptions about clinical trials. 38% Think it is about testing new drugs and even 58% think that children are not allowed to participate in clinical trials.



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Challenges in clinical trials in children

50% delayed due to patient recruitment

70% delayed by at least one month

70% failing to retain enough patients

In addition, there are many challenges in clinical trails in children. A very high percentage of trials is delayed due to patient recruitment or fails to retain the patients in the trials.

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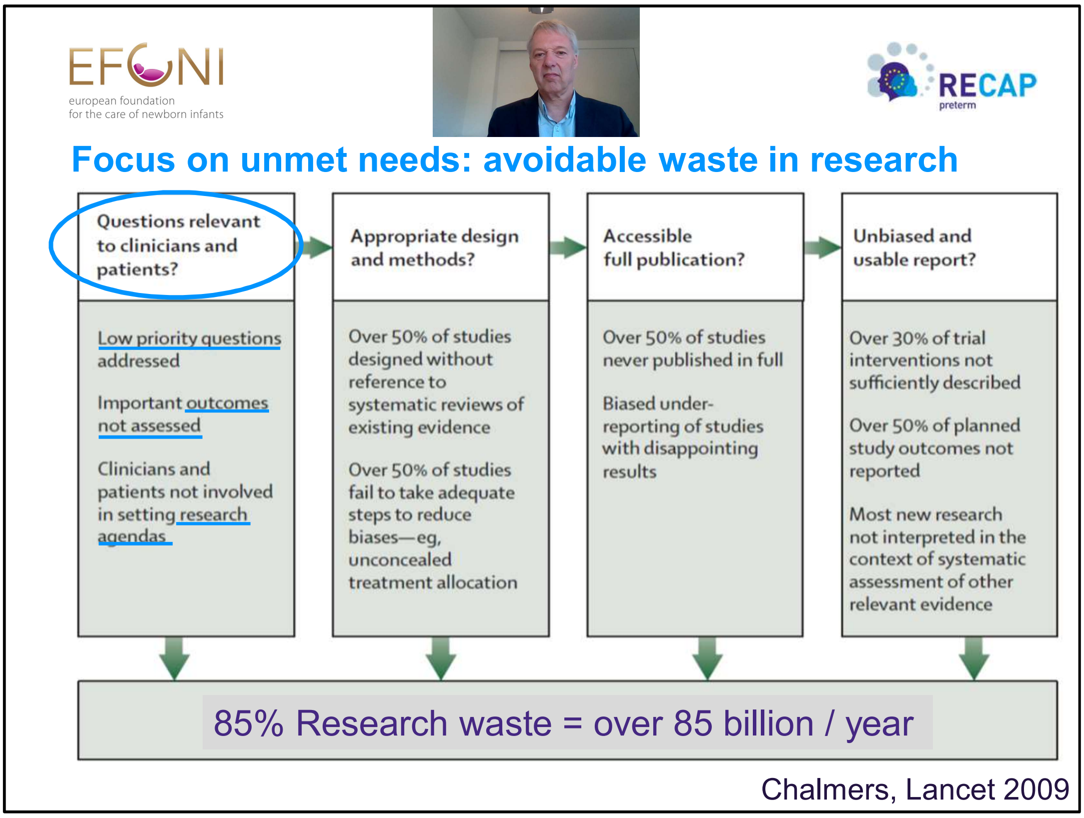
Involving patients in research projects: why would you?

- **Success** of research applications
- **Meaning of an illness** in daily lives
- **Research questions and outcomes matter to patients**
- **Acceptable** to patients and other stakeholders
- **Useful** for patients, and uptake in practice
- **Trust, credibility**
- **Dissemination** to broader lay audiences
- **New approaches, ideas or innovations**

This leads us to the question: why would you involve patients in your research projects? Well, it turns out that there are many advantages such the meaning, acceptability and usefulness of the research to patients, which makes it more successful and trustworthy. Patients can have new ideas and can play an important role in the dissemination.



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Focus on unmet needs: avoidable waste in research

Questions relevant to clinicians and patients?

Low priority questions addressed

Important outcomes not assessed

Clinicians and patients not involved in setting research agendas

Appropriate design and methods?

Over 50% of studies designed without reference to systematic reviews of existing evidence

Over 50% of studies fail to take adequate steps to reduce biases—eg, unconcealed treatment allocation

Accessible full publication?

Over 50% of studies never published in full

Biased under-reporting of studies with disappointing results

Unbiased and usable report?

Over 30% of trial interventions not sufficiently described

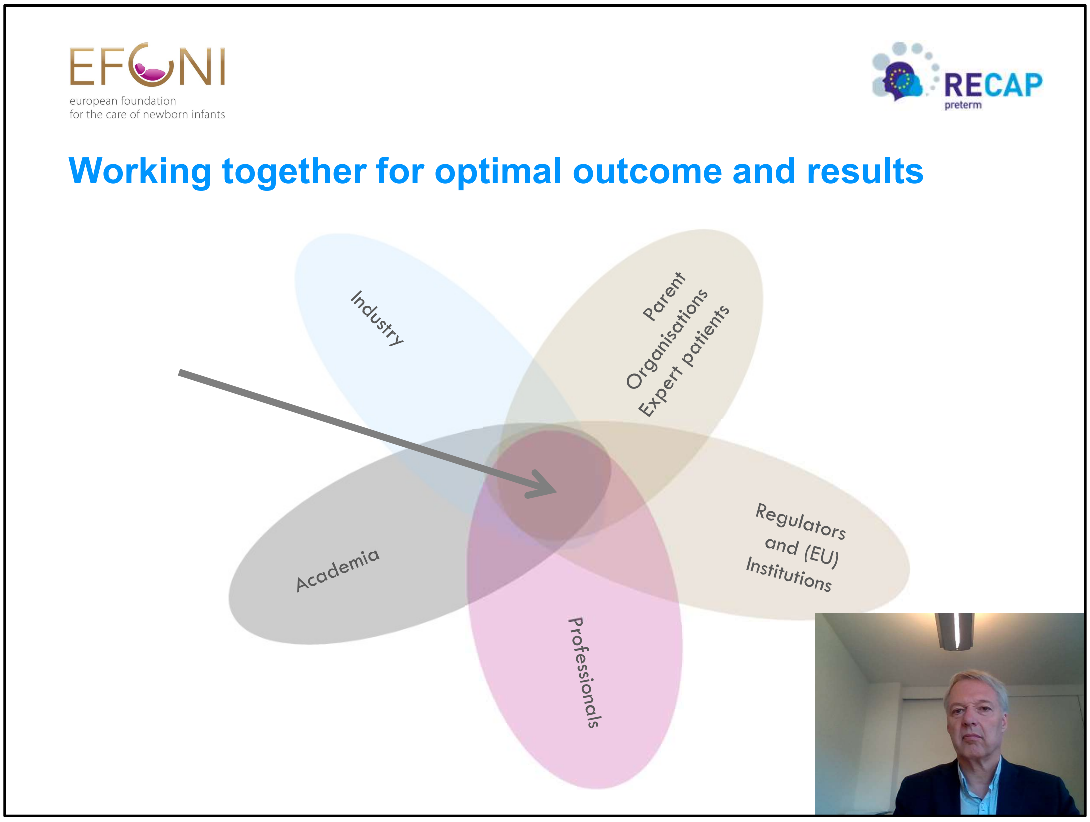
Over 50% of planned study outcomes not reported

Most new research not interpreted in the context of systematic assessment of other relevant evidence

85% Research waste = over 85 billion / year

Chalmers, Lancet 2009

In a Lancet article, Chalmers claims that 85% of the worlds yearly 100 billion spend on research is wasted. It can be avoided by a focus on unmet needs instead of investigating questions of low priority to clinicians and patients, or irrelevent outcomes. Involving patients can also help to focus design, push to publish the results and decrease bias.



Working together for optimal outcome and results

Industry

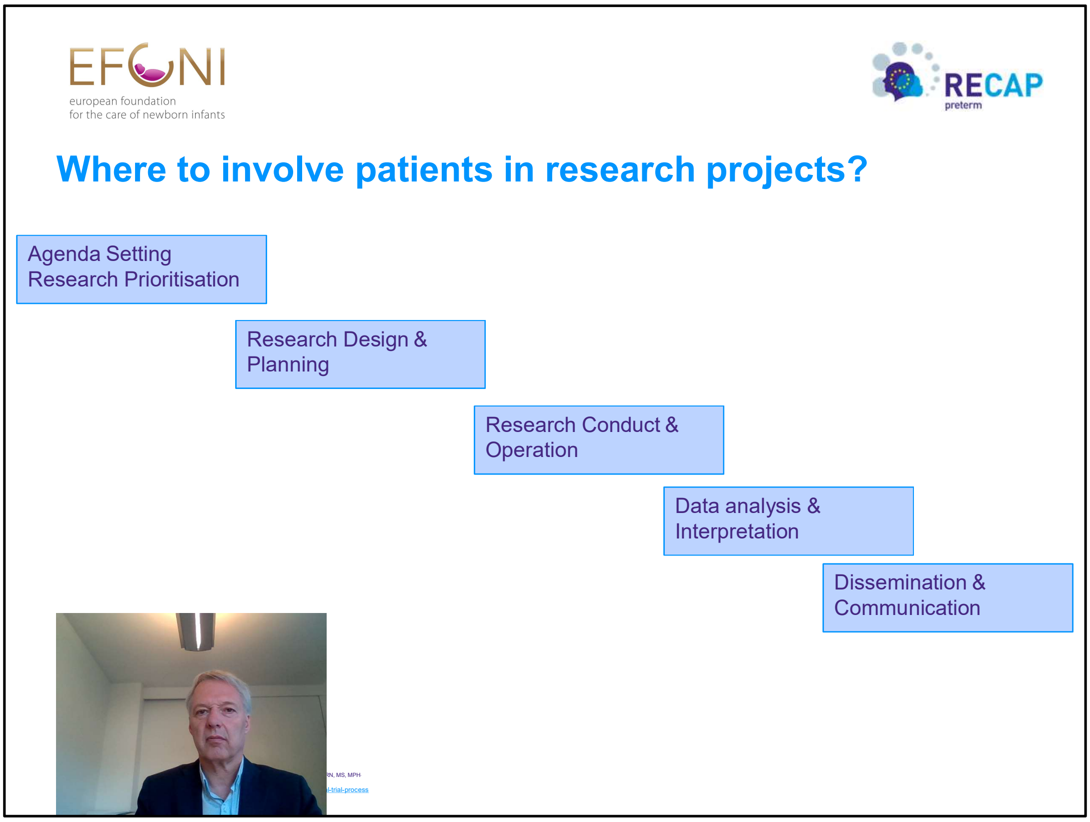
Parent
Organisations
Expert patients

Regulators
and (EU)
Institutions

Academia

Professionals

For optimal research, we need all stakeholders, from professionals and academia, to industry and regulators and also patient representatives. Aisling Walsh will later give an example of how to involve stakeholders in co-creation.



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Where to involve patients in research projects?

Agenda Setting
Research Prioritisation

Research Design & Planning

Research Conduct & Operation

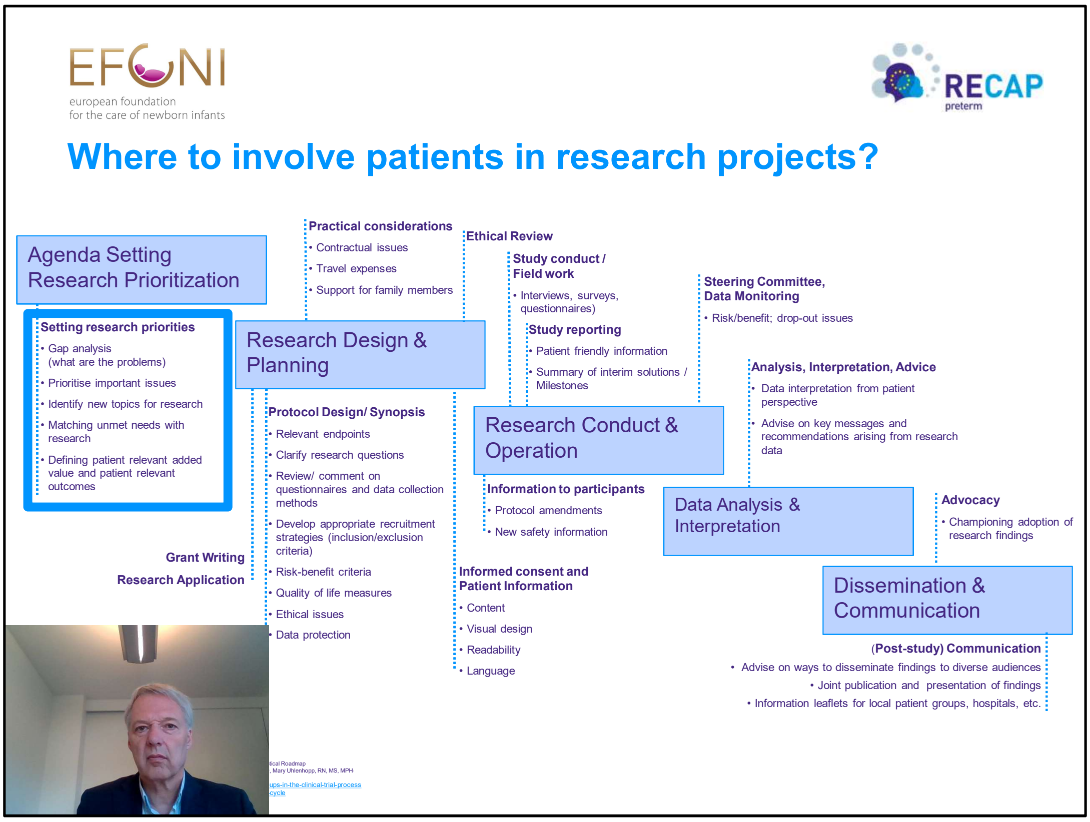
Data analysis & Interpretation

Dissemination & Communication

RN, MS, MPH

al-trial-process

But where then should we involve parents in research projects? Patients can and ideally should be involved at all stages of research projects from the early stages to the end. I already mentioned setting research priorities by asking the relevant questions. But also in designd of trials, the conduct, the data analysis and dissemination.



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Where to involve patients in research projects?

Agenda Setting
Research Prioritization

Setting research priorities
- Gap analysis (what are the problems)
- Prioritise important issues
- Identify new topics for research
- Matching unmet needs with research
- Defining patient relevant added value and patient relevant outcomes

Grant Writing
Research Application

Research Design & Planning

Practical considerations
- Contractual issues
- Travel expenses
- Support for family members

Protocol Design/ Synopsis
- Relevant endpoints
- Clarify research questions
- Review/ comment on questionnaires and data collection methods
- Develop appropriate recruitment strategies (inclusion/exclusion criteria)
- Risk-benefit criteria
- Quality of life measures
- Ethical issues
- Data protection

Ethical Review

Informed consent and Patient Information
- Content
- Visual design
- Readability
- Language

Research Conduct & Operation

Study conduct / Field work
- Interviews, surveys, questionnaires)

Study reporting
- Patient friendly information
- Summary of interim solutions / Milestones

Steering Committee, Data Monitoring
- Risk/benefit; drop-out issues

Information to participants
- Protocol amendments
- New safety information

Data Analysis & Interpretation

Analysis, Interpretation, Advice
- Data interpretation from patient perspective
- Advise on key messages and recommendations arising from research data

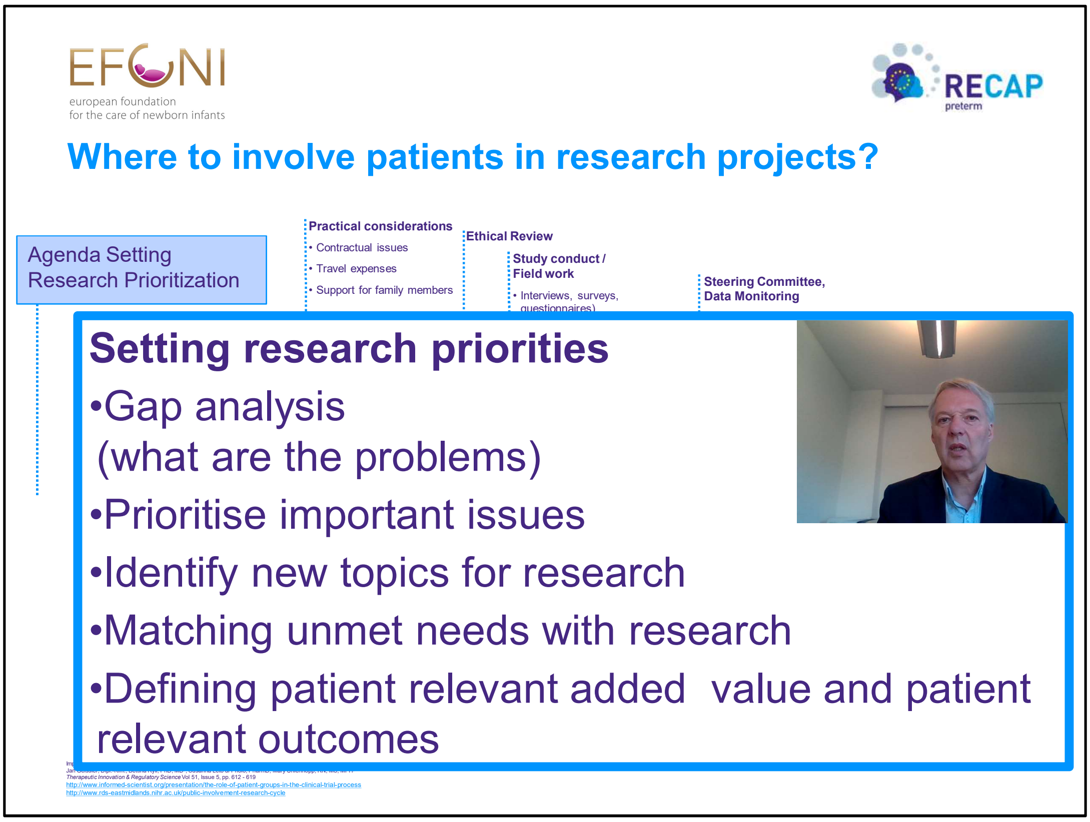
Dissemination & Communication

Advocacy
- Championing adoption of research findings

(Post-study) Communication
- Advise on ways to disseminate findings to diverse audiences
- Joint publication and presentation of findings
- Information leaflets for local patient groups, hospitals, etc.

tical Roadmap
, Mary Uhlenhopp, RN, MS, MPH
ups-in-the-clinical-trial-process
-cycle

This slide you don‘t have to read but it shows the multitude on items for possible involvement in more detail. Just two examples: defining relevant endpoinds during design and data interpretation from the patient perspective in data analysis. Lets now look in more detail at agenda setting.



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Where to involve patients in research projects?

Agenda Setting
Research Prioritization

Practical considerations
- Contractual issues
- Travel expenses
- Support for family members

Ethical Review

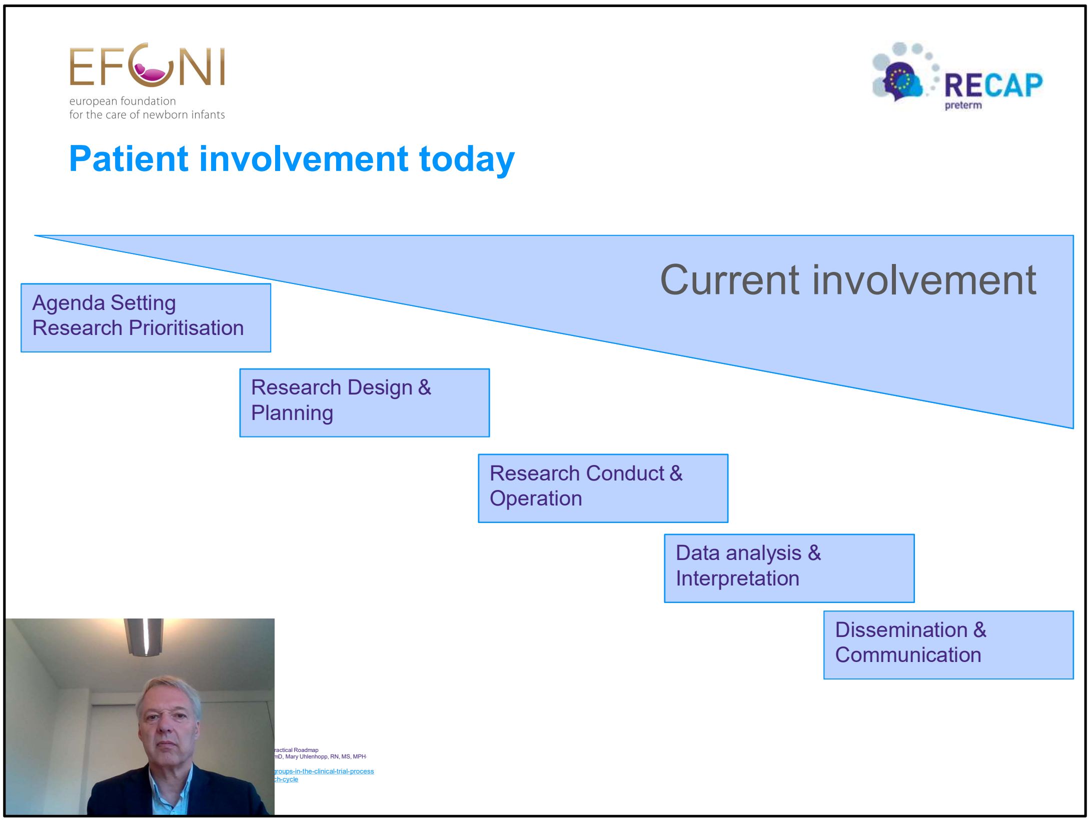
Study conduct / Field work
- Interviews, surveys, questionnaires)

Steering Committee, Data Monitoring

Setting research priorities
- Gap analysis (what are the problems)
- Prioritise important issues
- Identify new topics for research
- Matching unmet needs with research
- Defining patient relevant added value and patient relevant outcomes

Imp
Jan Geissler, Dipl.-Kfm., Bettina Ryll, PhD, MD, Susanna Leto di Priolo, PharmD, Mary Uhlenhopp, RN, MS, MPH
Therapeutic Innovation & Regulatory Science Vol 51, Issue 5, pp. 612 - 619
http://www.informed-scientist.org/presentation/the-role-of-patient-groups-in-the-clinical-trial-process
http://www.rds-eastmidlands.nihr.ac.uk/public-involvement-research-cycle

Patients can help in gap analysis and prioritise the problems, identify new topics and define patient relevant value and outcome.



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Patient involvement today

Current involvement

Agenda Setting
Research Prioritisation

Research Design &
Planning

Research Conduct &
Operation

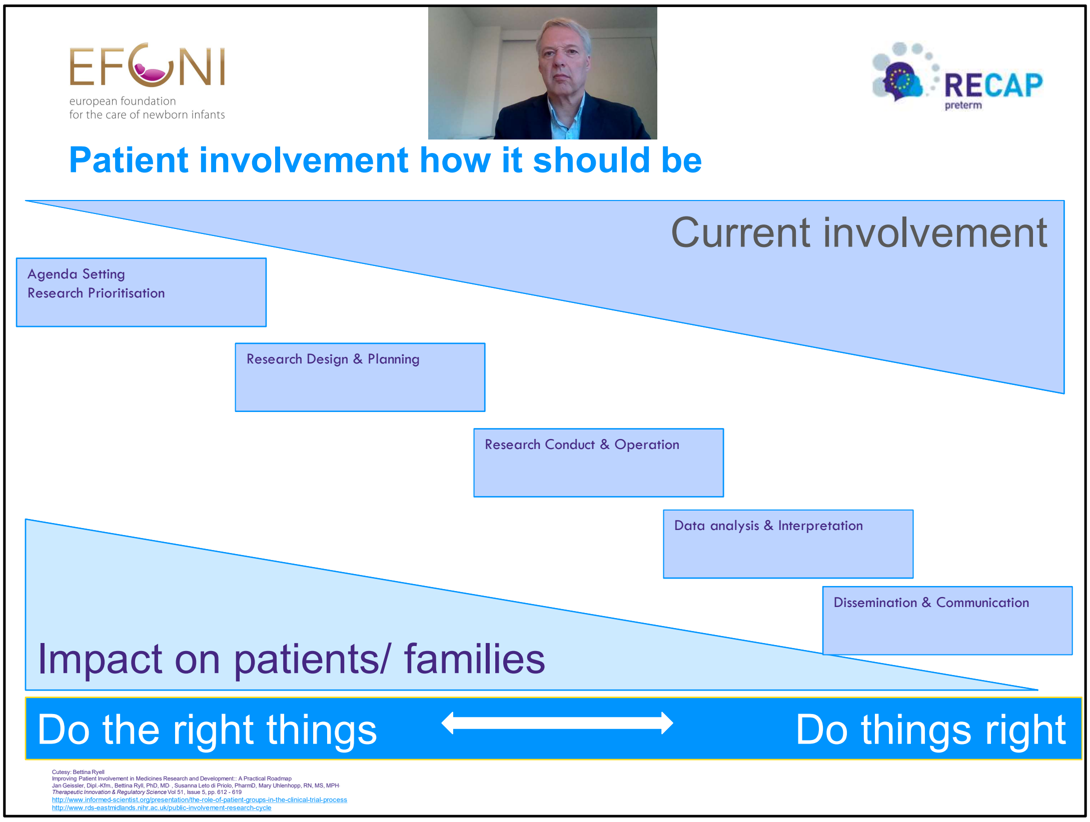
Data analysis &
Interpretation

Dissemination &
Communication

ractical Roadmap
mD, Mary Uhlenhopp, RN, MS, MPH

groups-in-the-clinical-trial-process
ch-cycle

Currently patients are mainly involved in the later stages of research, culminating in dissemination and communication, but much less in these important topics as agenda setting which we discussed.



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Patient involvement how it should be

Current involvement

Agenda Setting
Research Prioritisation

Research Design & Planning

Research Conduct & Operation

Data analysis & Interpretation

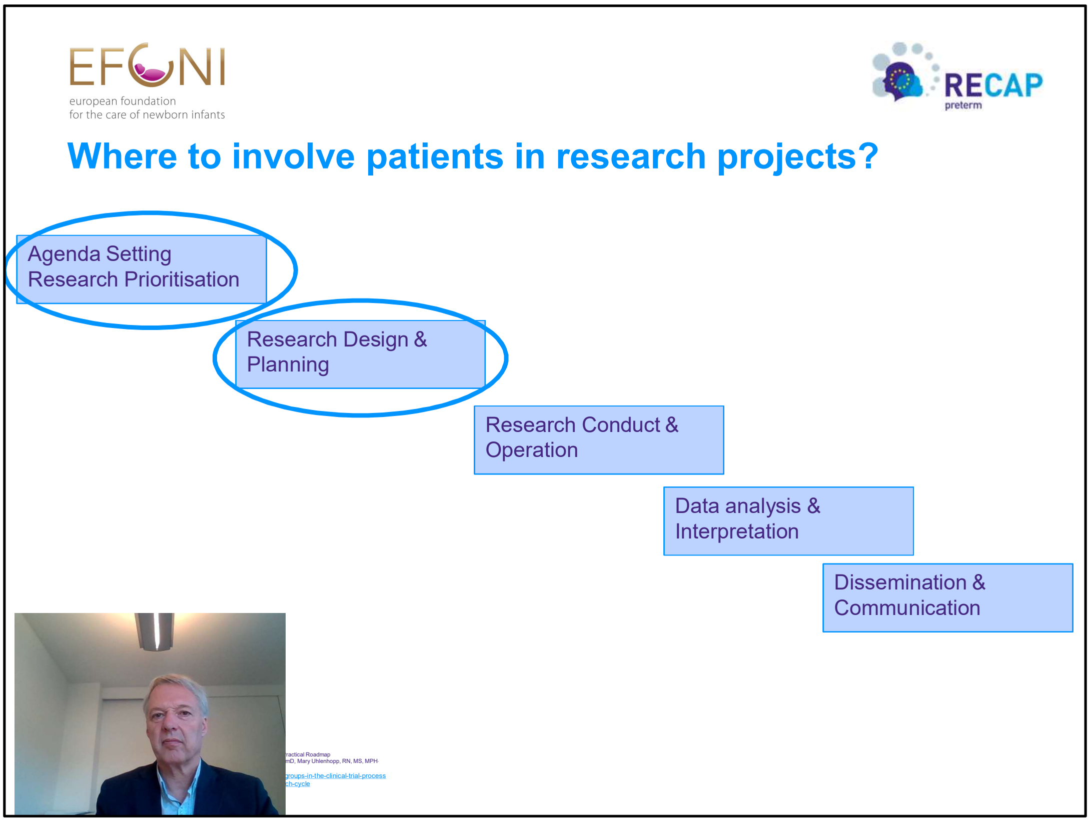
Dissemination & Communication

Impact on patients/ families

Do the right things ⟷ Do things right

Cutesy: Bettina Ryell
Improving Patient Involvement in Medicines Research and Development:: A Practical Roadmap
Jan Geissler, Dipl.-Kfm., Bettina Ryll, PhD, MD· , Susanna Leto di Priolo, PharmD, Mary Uhlenhopp, RN, MS, MPH·
Therapeutic Innovation & Regulatory Science Vol 51, Issue 5, pp. 612 - 619
http://www.informed-scientist.org/presentation/the-role-of-patient-groups-in-the-clinical-trial-process
http://www.rds-eastmidlands.nihr.ac.uk/public-involvement-research-cycle

However, as is clear from my previous arguments, the impact on patients and families is determined more by the early stages of research. This is where we want to involve them more in the future to ensure relevant research without waste. So, more focus on doing the right things instead of only doing things right!



Where to involve patients in research projects?

Agenda Setting
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Research Design &
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Research Conduct &
Operation

Data analysis &
Interpretation

Dissemination &
Communication

ractical Roadmap
mD, Mary Uhlenhopp, RN, MS, MPH

groups-in-the-clinical-trial-process
ch-cycle

In the next two slides I will give one example of involving patients in setting research priorities and in determining outcome during the study design phase.

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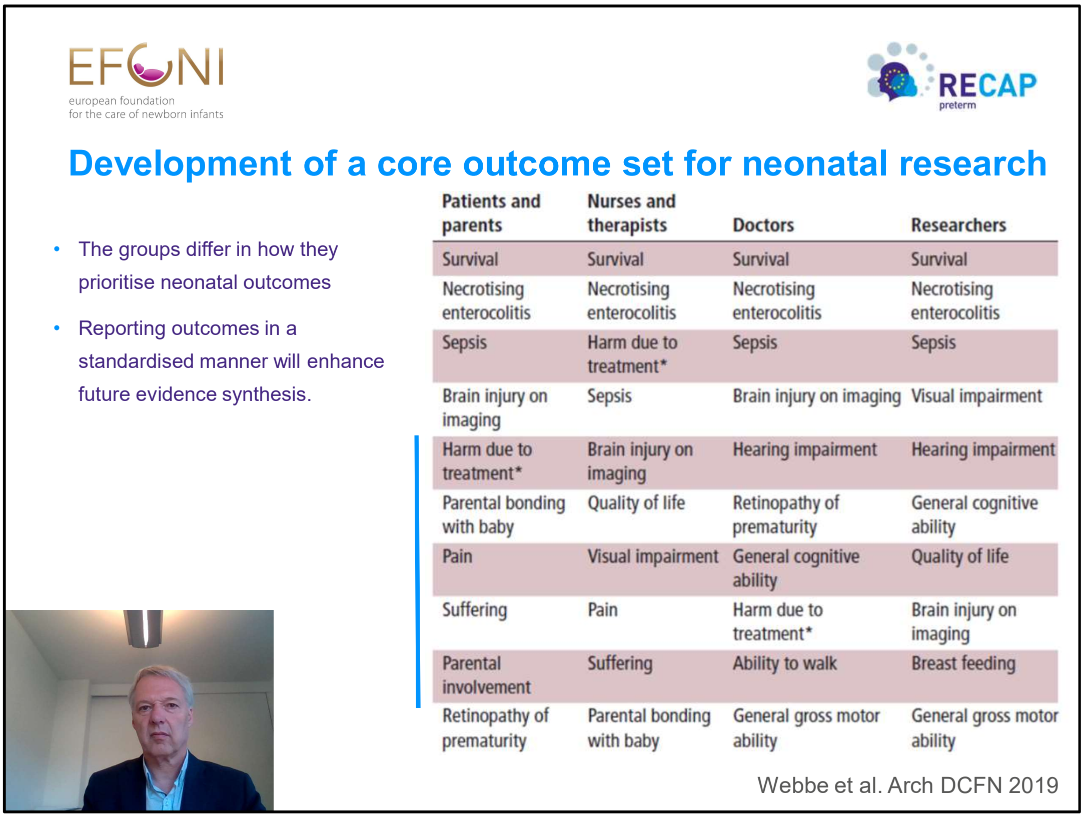
Top 15 UK research priorities for preterm birth (first 9 shown): developed with people affected by preterm birth and health care professionals

- How to **predict or prevent preterm birth**?
- How can **infection** be prevented?
- Which interventions to prevent **necrotizing enterocolitis**?
- What is the best treatment for **lung damage** in premature babies?
- What should be included to **support parents and families** when a premature baby is **discharged ?**
- What is the **optimum milk feeding strategy and guidance** for the best long-term outcomes?
- What is the best way to judge whether a premature baby **is feeling pain ?**
- Which treatments are most effective to **prevent early onset pre-eclampsia**?
- What emotional and practical support improves **attachment and bonding?**

Duley et a

The top 15 research priorities for preterm birth were developed in the UK with people affected by preterm birth and health care professionals together. The first four are the important medical outcomes, preterm birth itself, infections, NEC and lung damage. But beside these, we see that the next ones are influenced by and important to patients and families: support at discharge, optimum milk feeding strategy and guidance, judging pain, and attachment and bounding.



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Development of a core outcome set for neonatal research

- The groups differ in how they prioritise neonatal outcomes
- Reporting outcomes in a standardised manner will enhance future evidence synthesis.

Patients and parents	Nurses and therapists	Doctors	Researchers
Survival	Survival	Survival	Survival
Necrotising enterocolitis	Necrotising enterocolitis	Necrotising enterocolitis	Necrotising enterocolitis
Sepsis	Harm due to treatment*	Sepsis	Sepsis
Brain injury on imaging	Sepsis	Brain injury on imaging	Visual impairment
Harm due to treatment*	Brain injury on imaging	Hearing impairment	Hearing impairment
Parental bonding with baby	Quality of life	Retinopathy of prematurity	General cognitive ability
Pain	Visual impairment	General cognitive ability	Quality of life
Suffering	Pain	Harm due to treatment*	Brain injury on imaging
Parental involvement	Suffering	Ability to walk	Breast feeding
Retinopathy of prematurity	Parental bonding with baby	General gross motor ability	General gross motor ability

Webbe et al. Arch DCFN 2019

To study relevant outcomes and to standardise comparison between trials a recent trend is to define core outcome sets. This study by Webbe clearly shows how patients , parents and nurses differ in their priorities of outcomes from doctors and researchers. Again the very important medical complications of preterm birth scored highest in all groups: survival, NEC, sepsis and brain injury. However, harm due to treatment, parenteral bounding, pain, suffering and parental involvement were the items next on the list for patients and parents and mainly also for nurses, but hard medical outcomes continued to dominate for doctors and researchers.

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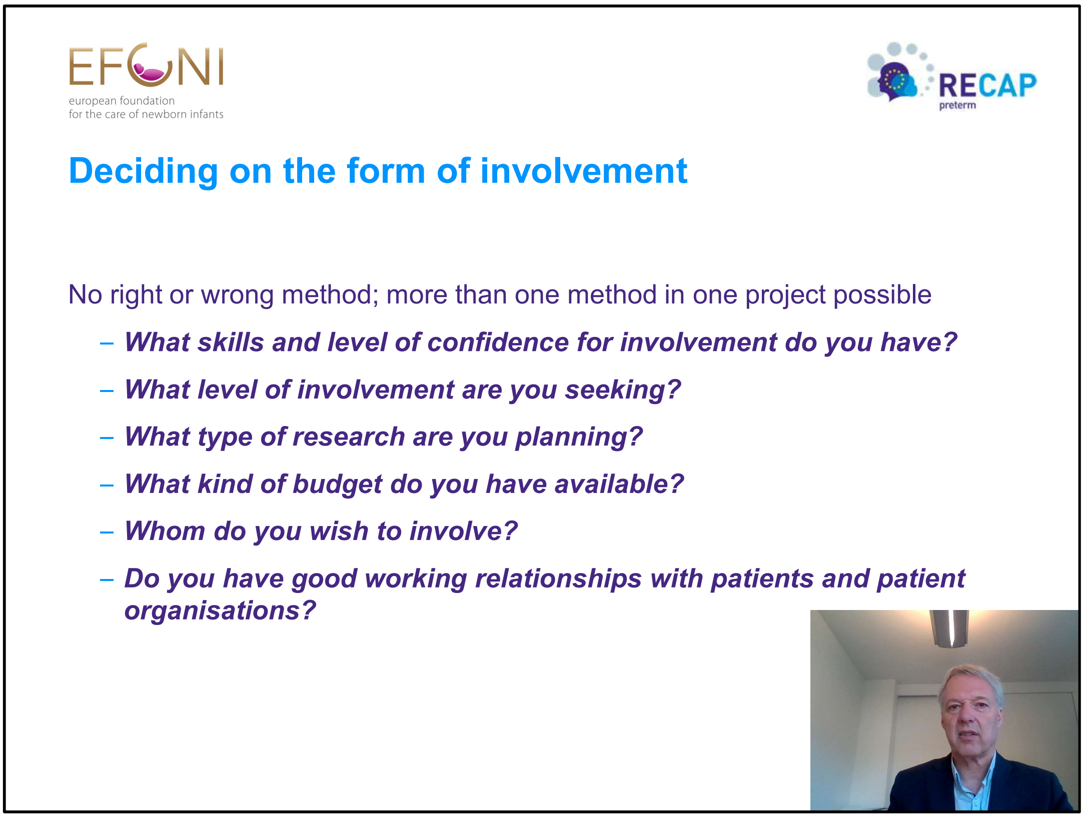
Patient involvement - from theory to practice?

Researchers as well as parents often lack the expertise to maximise the parents’ contributions

How can we realise a successful and meaningful patient involvement?

It is clear that at the moment researchers as well as parents often lack the expertise to maximise the parents’ contributions. How can we obtain this? I will only briefly introduce this, as this is discussed further in the next presentations.



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Deciding on the form of involvement

No right or wrong method; more than one method in one project possible

- ***What skills and level of confidence for involvement do you have?***
- ***What level of involvement are you seeking?***
- ***What type of research are you planning?***
- ***What kind of budget do you have available?***
- ***Whom do you wish to involve?***
- ***Do you have good working relationships with patients and patient organisations?***

It can be hard to decide which method to use to involve patients. there is no one right or wrong method for any project and

you may use more than one method. The following questions may help you to

identity which methods to use in your project:

What skills and level of confidence do you have?

if you have never involved patients before, think about using a method that you

feel confident about. For example, comments on patient information sheets or other

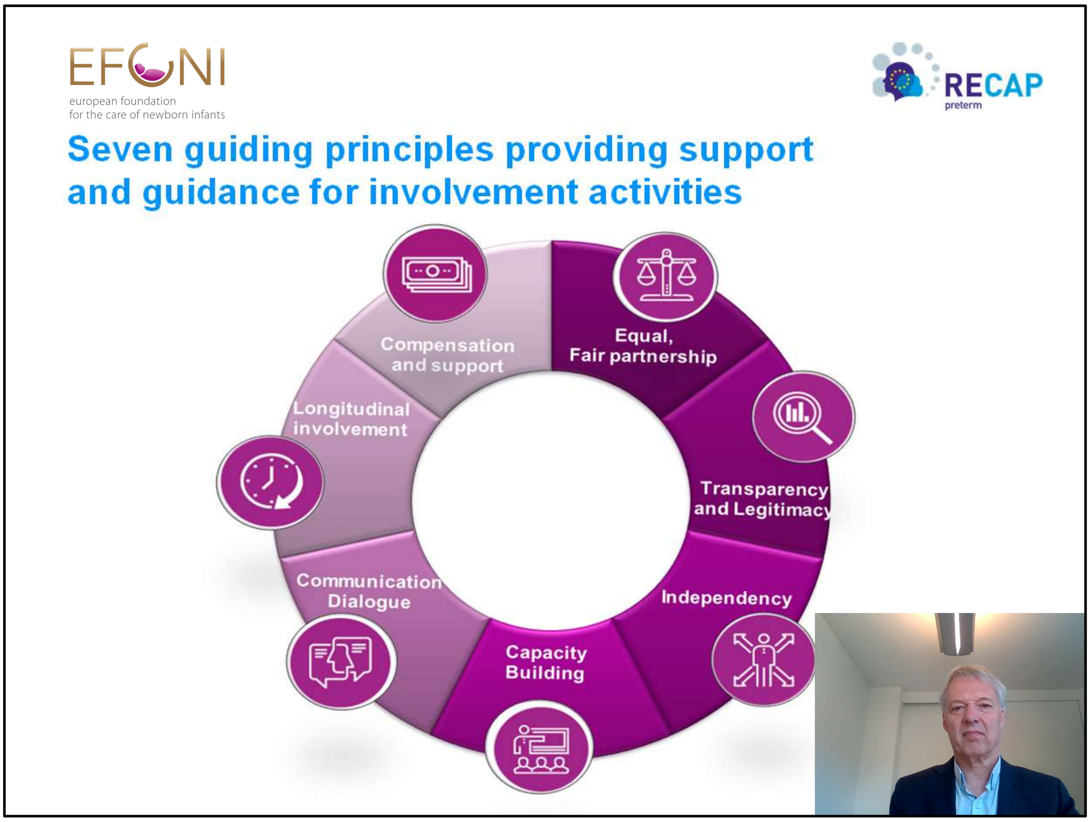
written materials.

What level of participation are you seeking and what type of research are you planning?

Of course the available budget can also determine your possiblities but it is important to plan budget for patient participation from the initial phases otherwise there will never be anything left over.

Who are you involving? And do you already have good working

relationships with patients and patient organisations?



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Seven guiding principles providing support and guidance for involvement activities

Equal,
Fair partnership

Transparency
and Legitimacy

Independency

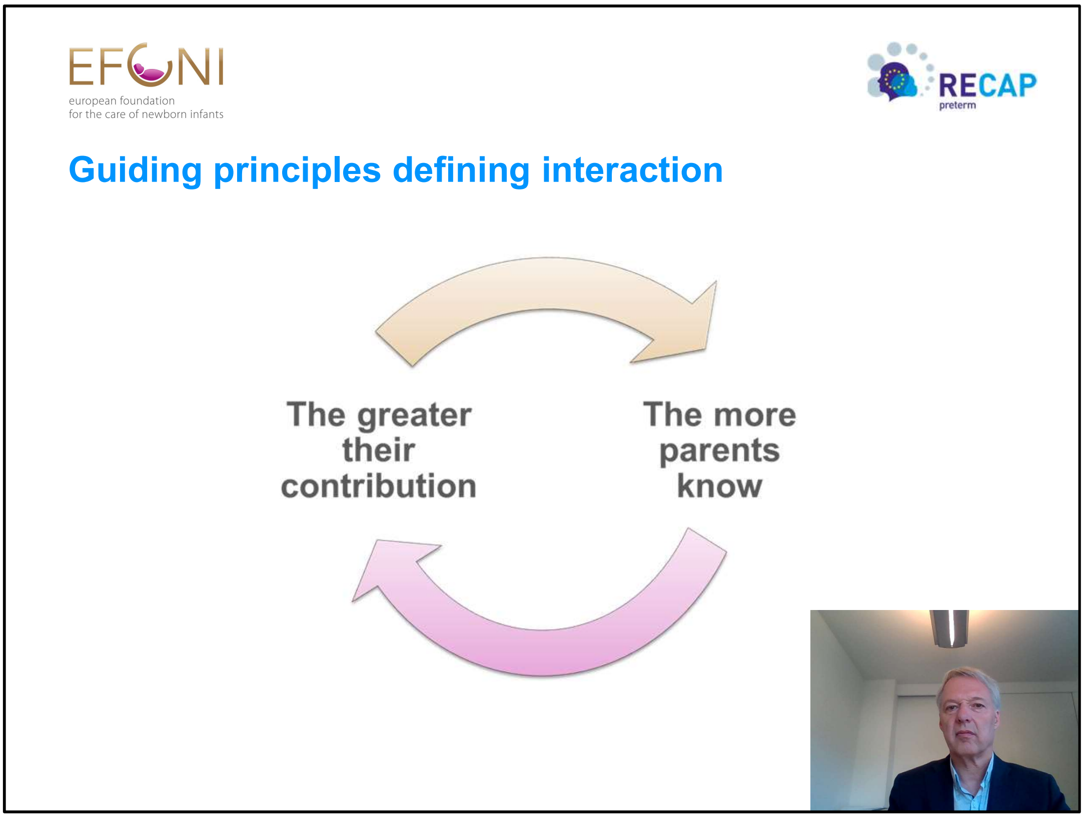
Capacity
Building

Communication
Dialogue

Longitudinal
involvement

Compensation
and support

These seven principles can guide you in the involvement of patients. Aisling Walsh and Nicole Thiele will expand on several of them. It is important to strive for equal and fair partnership, be transparant and clear from the beginning, build capacity trough training of both professionals and parents on how to involve patients and have attention to communication and the longitudinal aspects of the project.

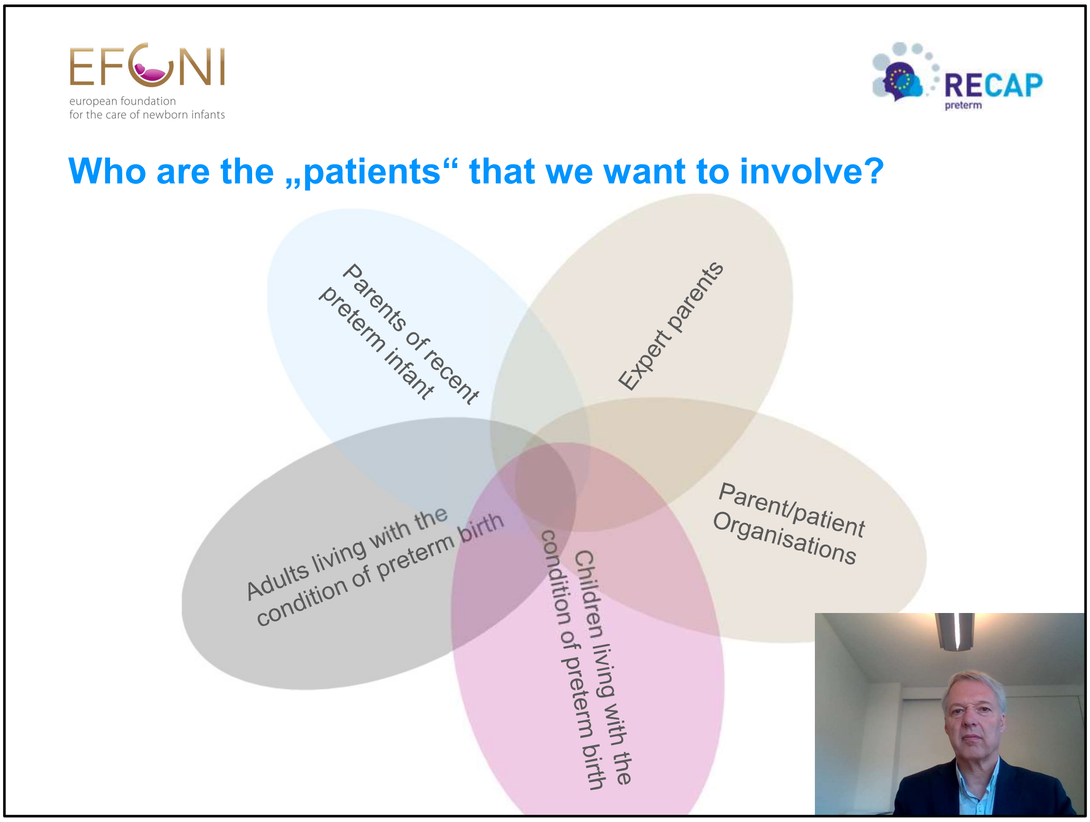


Guiding principles defining interaction

The greater their contribution

The more parents know

This summarises it nicely: The more patients or parents know the greater their contribution.



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Who are the „patients“ that we want to involve?

Parents of recent preterm infant

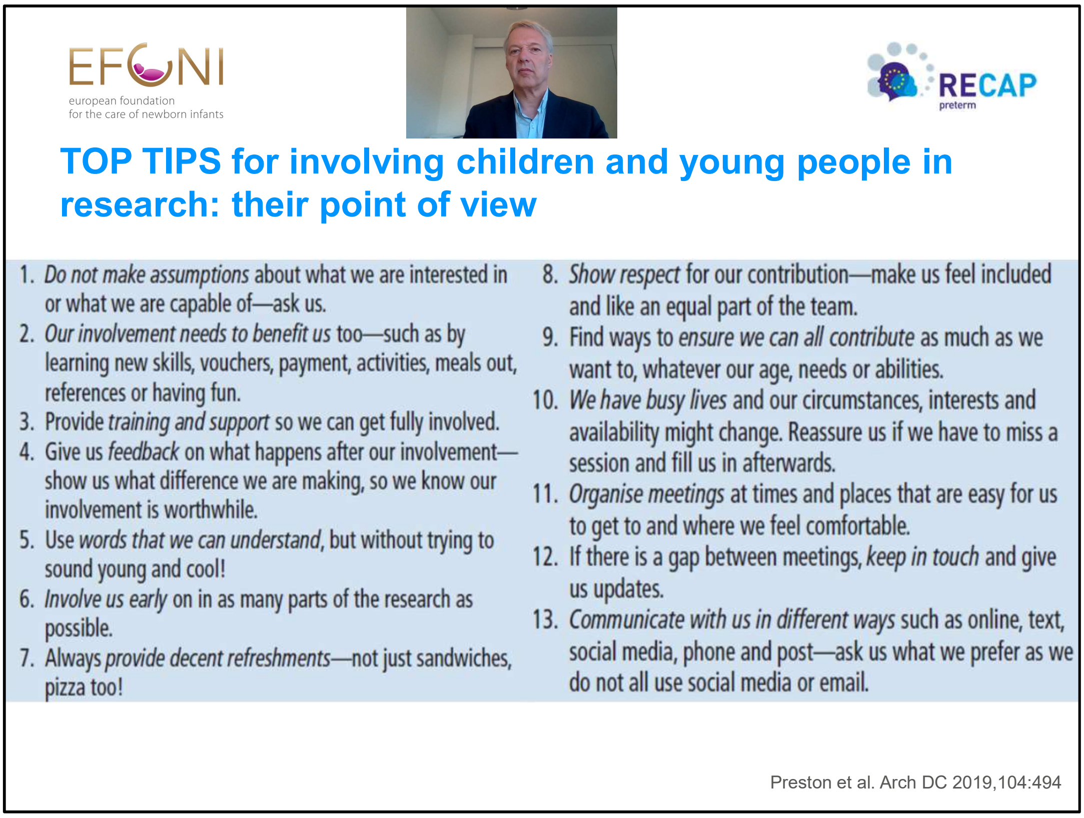
Expert parents

Parent/patient Organisations

Children living with the condition of preterm birth

Adults living with the condition of preterm birth

In my previous slides I switched between patients, parents, patient representatives and others. Before I finish, I want to clarify who we can involve as patient representatives. Parents of a recent preterm infant have up to date experiences which provide very useful feedback and ideas, expert parents have already gained experience in contributing to research and other topics and can add more advanced aspects. Parent organisations have the network to contact parents and other stakeholders, have more expertise, can advocate and disseminate. They can represent a small or wide spectrum of patients or consist of an umbrella organisation having a network in for ex. Europe and beyond, such as EFCNI. Finally, and especially important for long term follow-up studies, are adults living with the condition of preterm birth and in the future, also children should be more involved. So all are important and have specific benefits and characteristics for involvement



TOP TIPS for involving children and young people in research: their point of view

1. *Do not make assumptions* about what we are interested in or what we are capable of—ask us.
2. *Our involvement needs to benefit us* too—such as by learning new skills, vouchers, payment, activities, meals out, references or having fun.
3. Provide *training and support* so we can get fully involved.
4. Give us *feedback* on what happens after our involvement—show us what difference we are making, so we know our involvement is worthwhile.
5. Use *words that we can understand*, but without trying to sound young and cool!
6. *Involve us early* on in as many parts of the research as possible.
7. Always *provide decent refreshments*—not just sandwiches, pizza too!
8. *Show respect* for our contribution—make us feel included and like an equal part of the team.
9. Find ways to *ensure we can all contribute* as much as we want to, whatever our age, needs or abilities.
10. *We have busy lives* and our circumstances, interests and availability might change. Reassure us if we have to miss a session and fill us in afterwards.
11. *Organise meetings* at times and places that are easy for us to get to and where we feel comfortable.
12. If there is a gap between meetings, *keep in touch* and give us updates.
13. *Communicate with us in different ways* such as online, text, social media, phone and post—ask us what we prefer as we do not all use social media or email.

Preston et al. Arch DC 2019,104:494

One example from the viewpoint of children in involvement in research projects:

I just read a few.

1. Do not make assumptions about what we are interested in or what we are capable of- ask us!

5. Use words that we can understand, but without trying to sound young and cool

6. Invove us early

8. Show respect for our contribution

13. Communicate with us in diffrent ways such as online, text, social media...

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Take home message

- The **active patient partner** has become an acknowledged concept to shape our future healthcare and research
- Including patients/parents as equal research partners from the **very beginning and in all research phases** has many benefits
- Despite good examples, patient involvement across the research cycle **lacks structure and consistency** (“when”, how“ to involve patients)
- The **“7 guiding principles”** are a useful tool to guide patient involvement, e.g.
  - Be very clear from the beginning about role, tasks, timelines, budget
  - Training for researchers to effectively include patients in research projects
- Don‘t just involve **parents or parent organisations but also patients** living with the condition of preterm birth

Lets conclude:

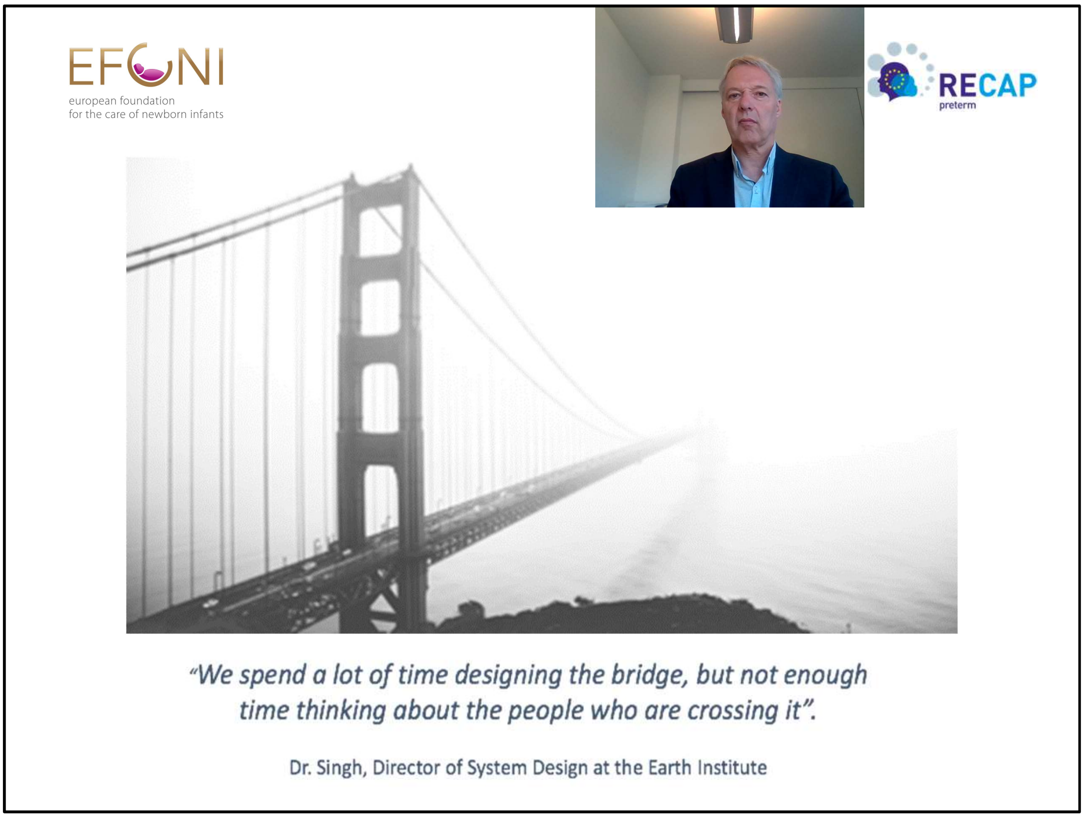
The **active patient partner** has become an acknowledged concept to shape our future healthcare and research.

Ensure that tey are equal partners involved from the **very beginning and in all research phases.** This has shown to have many benefits.

Despite good examples, patient involvement lacks **structure and consistency** (“when”, how“ to involve patients)

The **“7 guiding principles”** are a useful tool to guide patient involvement. Two are to be clear from the beginning and train researchers and patients.

And last but not least: Don‘t just involve **parents or parent organisations but also patients** living with the condition of preterm birth



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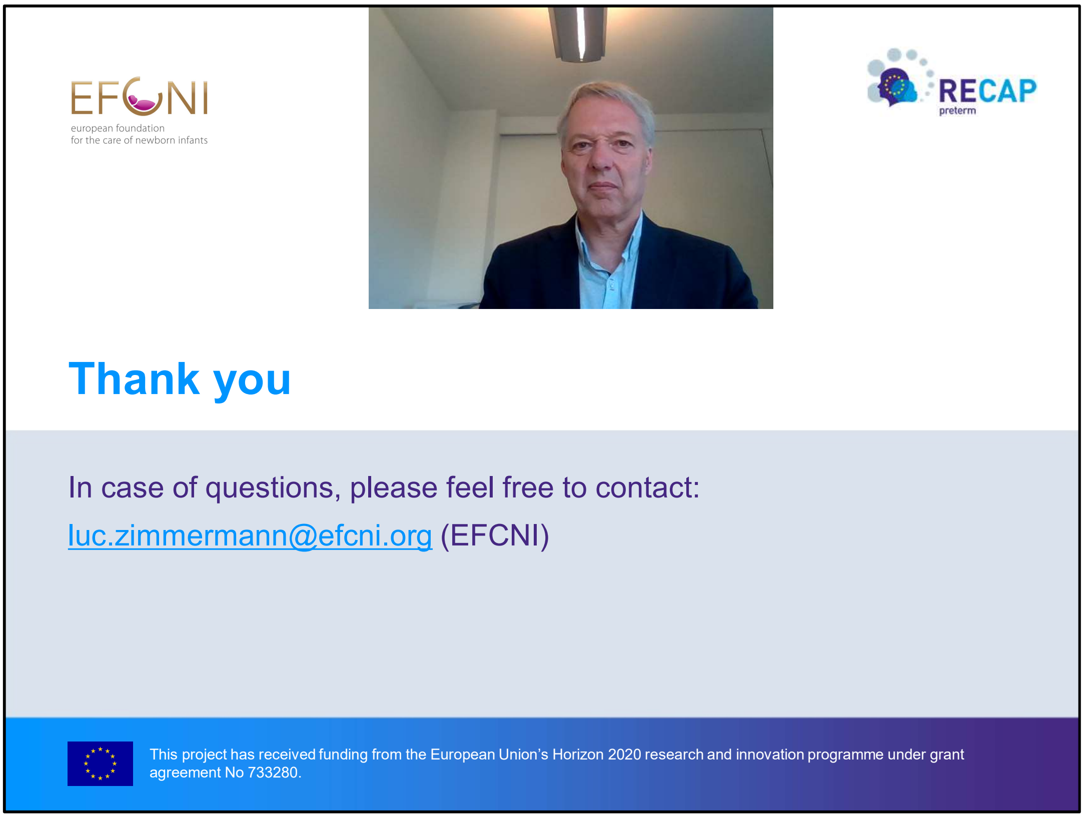
"We spend a lot of time designing the bridge, but not enough time thinking about the people who are crossing it".

Dr. Singh, Director of System Design at the Earth Institute

This also holds for our research: We spend a lot of time designing the bridge, but not enough time thinking about the people who are crossing it.



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Thank you

In case of questions, please feel free to contact:
luc.zimmermann@efcni.org (EFCNI)

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Thank you for your attention and keep up excellence in research!