



FIRST KEY FINDINGS FROM THE INTERNATIONAL SARCOMA PRIORITY SETTING PARTNERSHIP SURVEY

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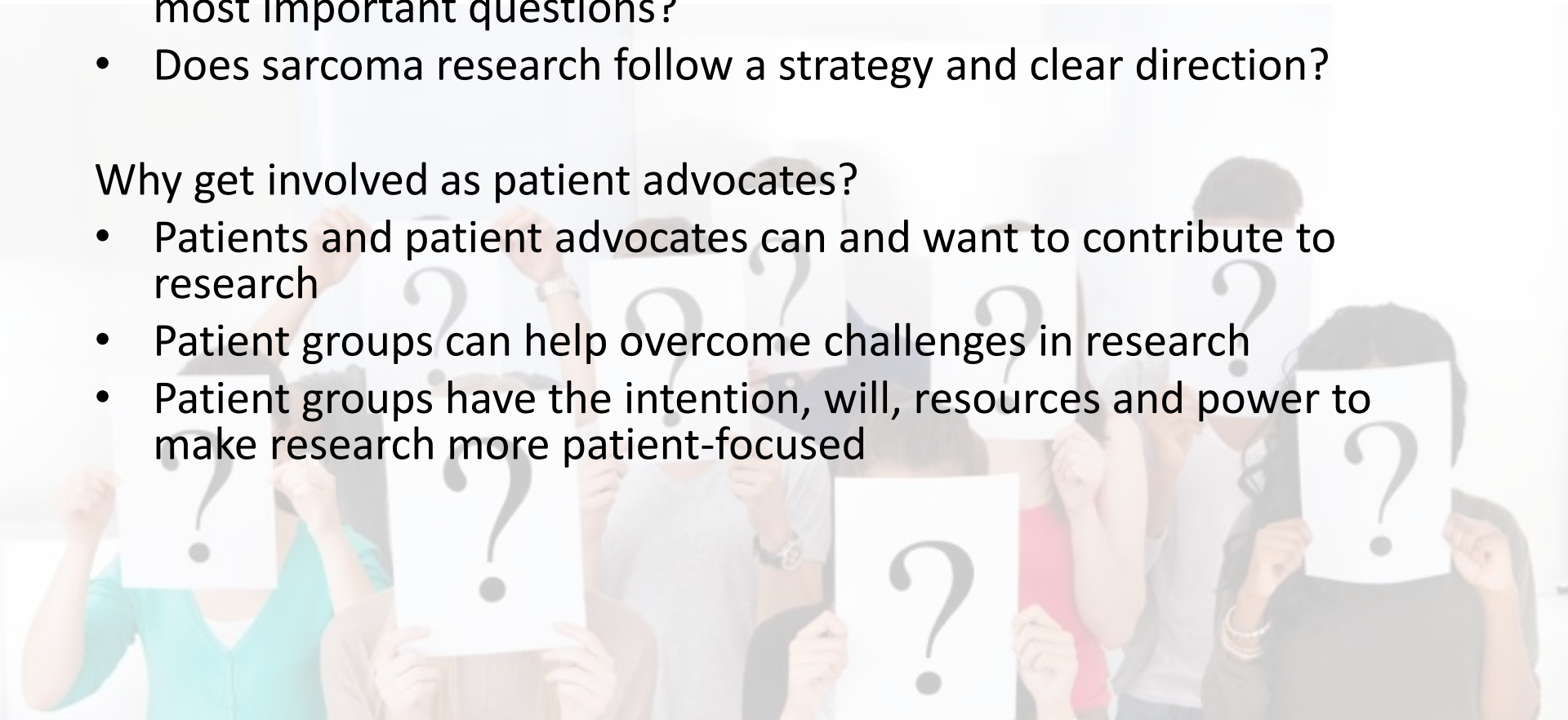
Why this all started

Our questions:

- What does sarcoma research decision making depend on?
- Does research in sarcomas answer actual patients' concerns and most important questions?
- Does sarcoma research follow a strategy and clear direction?

Why get involved as patient advocates?

- Patients and patient advocates can and want to contribute to research
- Patient groups can help overcome challenges in research
- Patient groups have the intention, will, resources and power to make research more patient-focused



Scientific background: Patient-centered care

- Patients are gaining a more active role in health care systems
- *Providing care that is respectful of and responsive to individual patient preferences, needs, and values, and ensuring that patient values guide all clinical decisions*



- Doctor–patient relationships are changing: shared decision making and patient empowerment are at the front of care.

- Patient advocacy groups state that their opinions must have greater influence on the decisions that affect them: “nothing about me without me”.



- Patient-centered care cannot be practiced without patients participating in their own health care decisions and in the research that informs such decisions

Patient-centered research?

- Cultural shift in health care is not having the same impact on the research process
- Predominant culture: research is performed on patients, not with patients (source of data and not as the true protagonists in the process)
- Initiatives have been developed in recent years to change this situation: Patient and Public Involvement (PPI) in research.

What can patients and the public offer research?

Patients, service users, carers and members of the public:

- improve the quality and relevance of research;
- make sure research is acceptable and will benefit patients, carers and service users; and
- support researchers and healthcare professionals to develop and carry out high-quality, well designed research studies.

As well as helping to make sure research is acceptable, of high quality and relevant, you also **have the right** to be involved in decisions about research which is funded by **public money**.

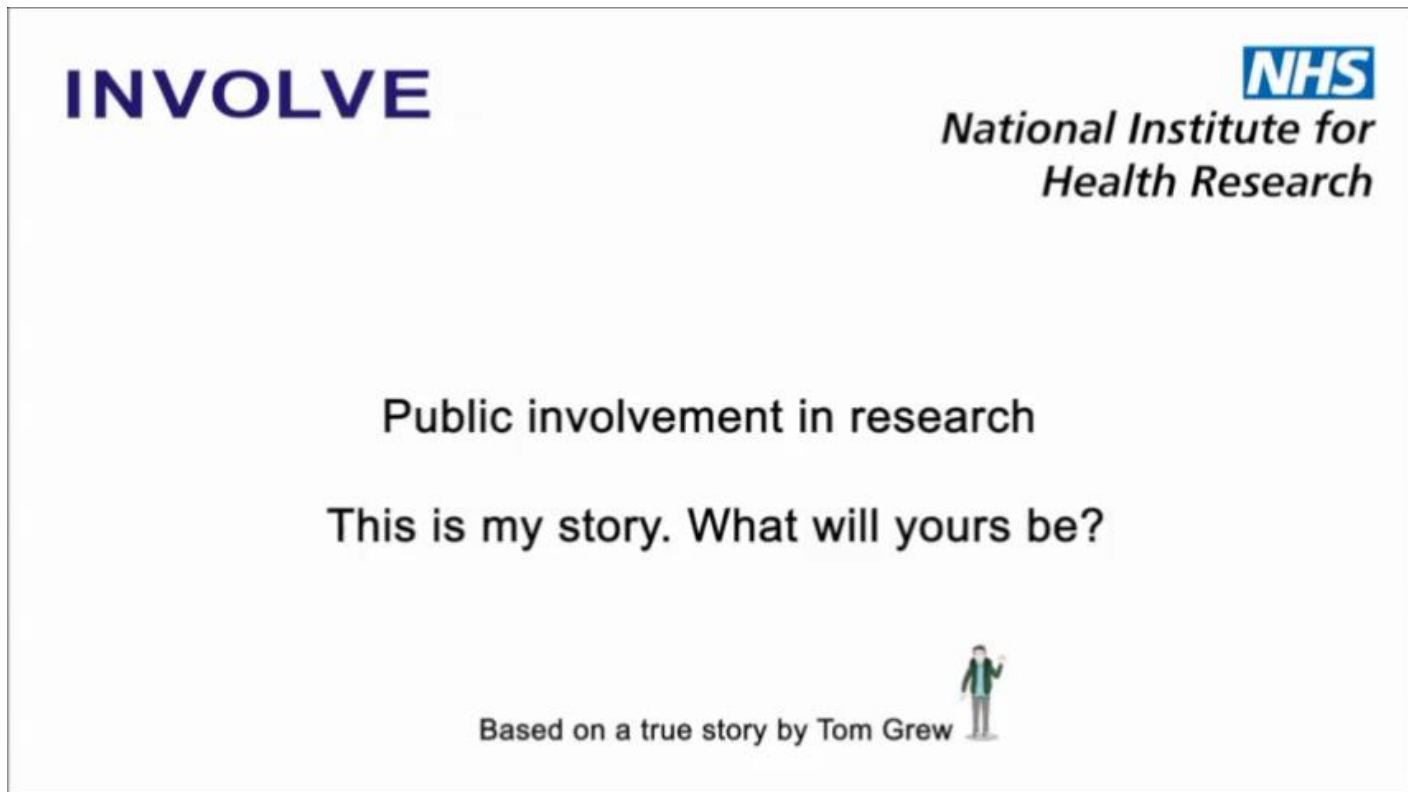
“No matter how complicated the research, or how brilliant the researcher, patients and the public always offer **unique, invaluable insights**. Their advice when designing, implementing and evaluating research invariably makes studies **more effective, more credible and often more cost-effective.**”



Professor Dame Sally Davies (Chief Medical Officer and our founder NIHR)

Why do people get involved in research?

The following animation gives an insight into why someone might get involved



Patient and public involvement (PPI)

Involvement



Research which is done **with** or **by** patients and the public, rather than **to**, **for** or **about** them. It is an active partnership between researchers and patients and the public. Patients and the public are involved in key decisions throughout the research project life cycle.

Participation



Where people give their informed consent to take part in a research study. For example, take trial drugs, try a new procedure or type of care, fill in questionnaires or be interviewed about their experiences. They are usually called study participants.

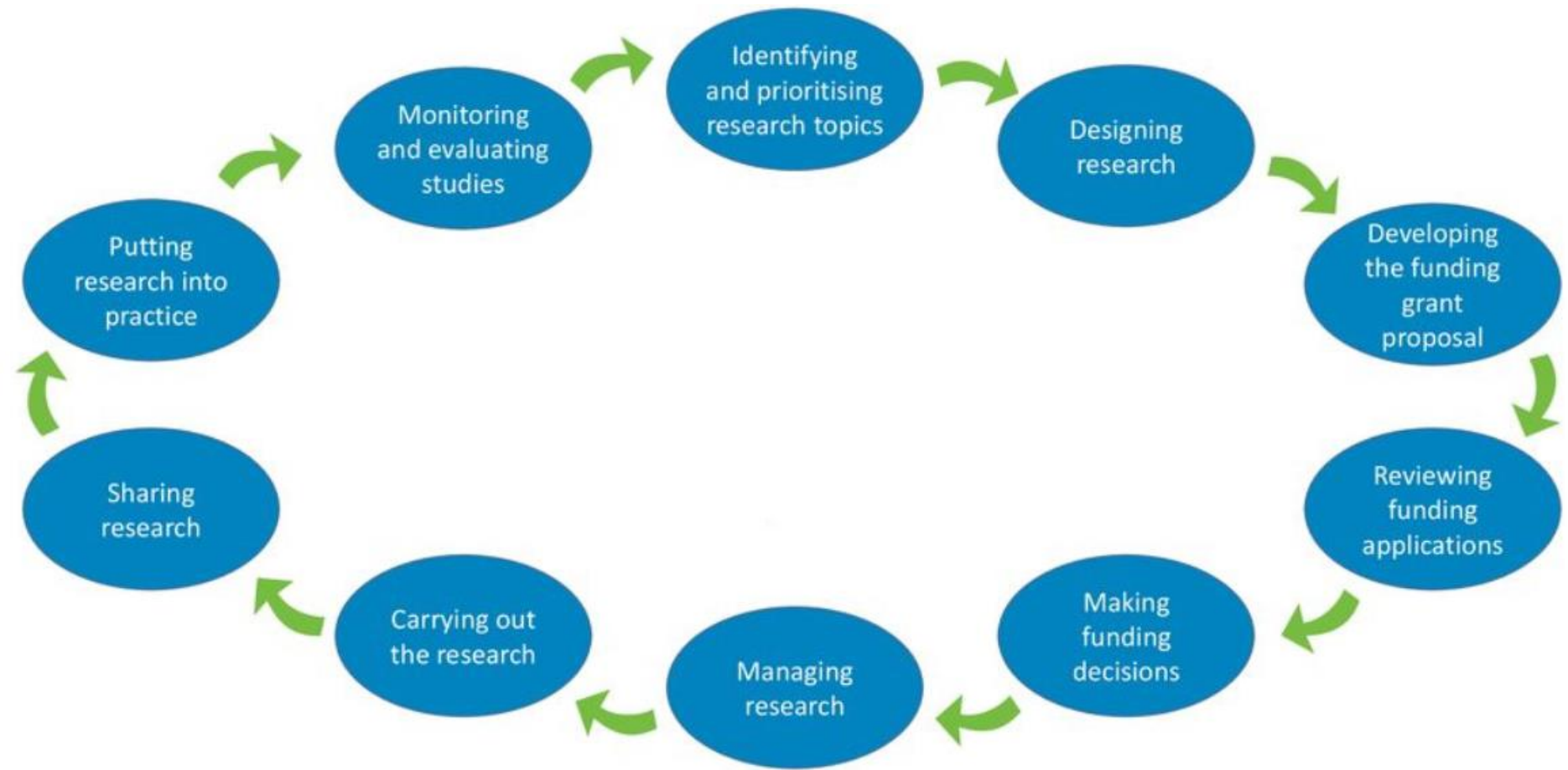
Engagement



Where we share information and knowledge about research with the public who we listen to and learn from as part of the process. The public can ask questions and debate results of the research.

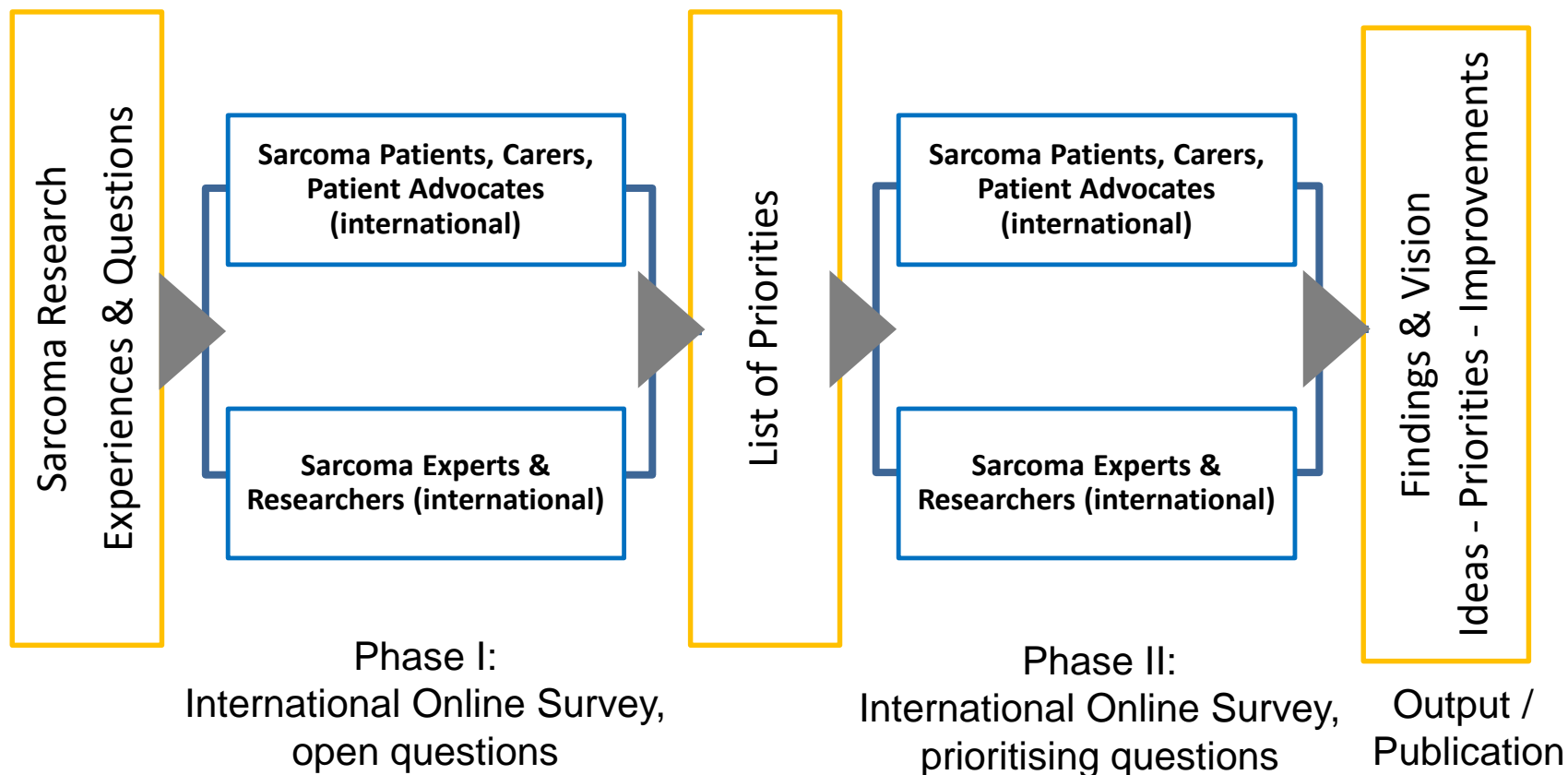
All three ways of working with patients and the public are important. Involvement adds to and improves research, and research is not possible without people taking part.

Roles in the research project life cycle



Aims SPAEN project

- Examine how sarcoma patients and carers want to be **involved** in research
- Determine the **research priorities** of sarcoma patients and carers



Steps

Phase 1

- Step 1: Identification and invitation of potential partners
- Step 2: Awareness raising
- Step 3: Identifying evidence uncertainties & ways to involve patients and carers in research

Phase 2

- Step 4: Refining questions/uncertainties and ways to involve patients and carers in research
- Step 5: Prioritisation

Steps

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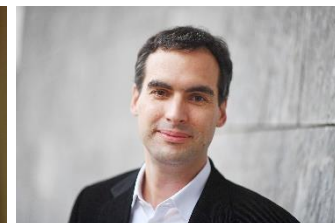
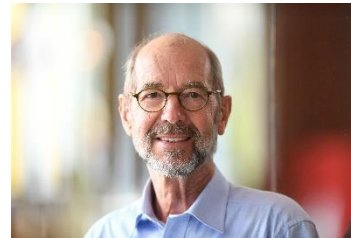
Phase 2

- Step 4: Refining questions/uncertainties and ways to involve patients and carers in research
- Step 5: Prioritisation

- March 27 2019: A meeting in Amsterdam (Netherlands Cancer Institute) was held and attended by SPAEN (Kathrin Schuster, Markus Wartenberg, Gerard van Oortmerssen) and Olga Husson and Winette van der Graaf.
- April 18 2019: The first steering group meeting was held over phone to discuss the aims, objectives and scope of the project

Steering group members

- Richard Davidson, UK
- Natalia Fernandez, Spain
- Winette van der Graaf, The Netherlands/UK
- Phil Green, UK
- Olga Husson, The Netherlands/UK
- Bernd Kasper, Germany
- Gerard van Oortmerssen, The Netherlands
- Kai Pilgermann, Germany
- Sami Sandakly, France
- Kathrin Schuster, Germany
- Lotta Våde, Norway
- Roger Wilson, UK



Steps

Phase 1

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- **Step 2: Awareness raising**
- Step 3: Identifying evidence uncertainties & ways to involve patients and carers in research

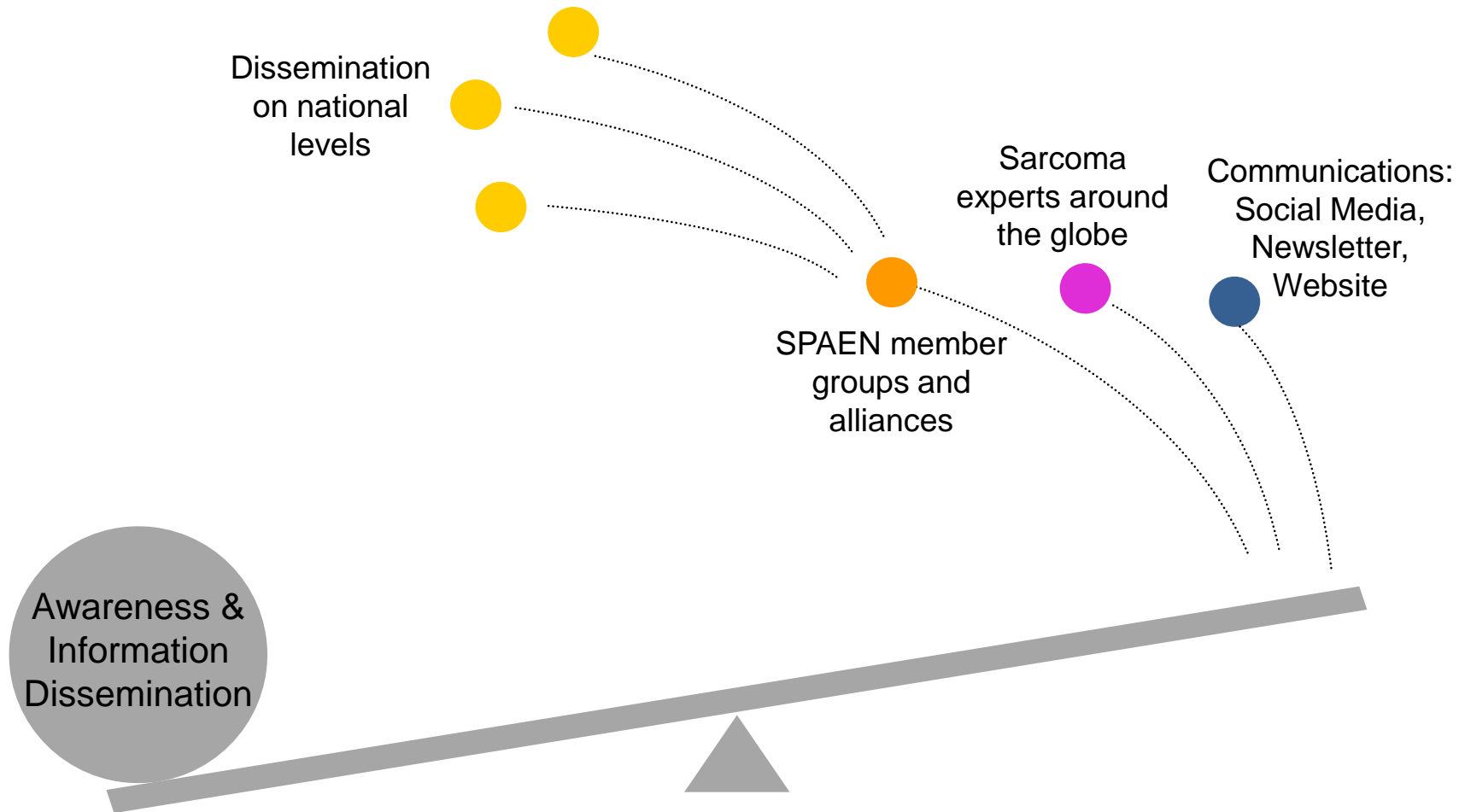
Phase 2

- Step 4: Refining questions/uncertainties and ways to involve patients and carers in research
- Step 5: Prioritisation

Awareness raising & Information Dissemination

- Information dissemination
- SPAEN Member groups & alliances → spread on national level
- Sarcoma experts around the globe
- Twitter
- Facebook

Awareness Raising & Information Dissemination



Steps

Phase 1

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- Step 2: Awareness raising
- **Step 3: Identifying evidence uncertainties & ways to involve patients and carers in research**

Phase 2

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Methods

Steering group developed the:



Sarcoma Priority Setting Partnership Survey

Have you ever had a question about the diagnosis, treatment, survivorship or end-of-life care of sarcoma and not been able to find out the answer? Are there any aspects of sarcoma which you feel should be addressed by research? If so, please take part in this survey.

Why we need your help?

We want to know what needs to be improved about the diagnosis, treatment and care of people living with or beyond sarcoma. We want to use your questions to help set priorities for research. This means: Your experience with this disease will help us understand where research is needed that will make a difference to people's lives.

Who could get involved?

- a person with sarcoma (including gastrointestinal stromal tumors (GIST) and desmoid tumors)
- a sarcoma survivor or person who once had sarcoma
- a carer for, or family member of, someone with sarcoma
- a bereaved carer or family member of someone who had sarcoma
- a healthcare professional working with people with sarcoma
- an organisation representing the interests of people with sarcoma
- a researcher focusing on sarcoma

Survey - Section 1 Research priorities

What question(s) about “.....” would you like to see answered by research?

- diagnosis of sarcoma
- treatment(s)/treatment decisions
- support
- health-related quality of life issues and symptoms
- survivorship/ clinical follow-up would you like to see answered by research?
- end of life issues

Survey – Section 2 Research involvement

- Have you ever been asked to take part in sarcoma research?
- Have you ever been involved in sarcoma research?
- Do you want to be involved in research?
- Patients/carers can contribute meaningfully to the research process
- How do you think patients/carers can contribute to the research process?
- Where do you think patients/carers can make an impact?
- Why do you think patients/carers do or don't take part in research?
- What could help patients/carers to become involved?

Survey – Section 3 About you

Sociodemographic and clinical characteristics of participants

Official launch May 2019



SPAEN @sarcomapatient · 9 aug. 2019

Anybody who's affected by [#sarcomas](#): Please help us identify the top 10 unanswered questions you want sarcoma research to address. This will help guide sarcoma research!

Share your thoughts & fill out our survey:
bit.ly/SarcSurvey

[#sarcoma](#) @Winette_vdGraaf @OlgaHusson



Preliminary results

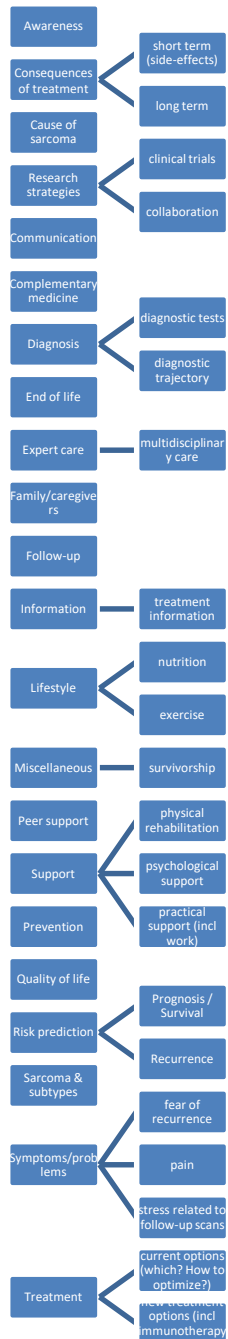
- Patients & carers (N=264)
- Health care professionals & researchers (N=48)

Patient and carer characteristics

- 76 male (29%); 183 female (69%); 5 missing
- Median age 53 years (range 19-82y); 5 missing
- 193 patients (73%); 50 carers (19%); 11 bereaved carers (4%); 8 other (3%); 2 missing
- 31 bone sarcoma (12%); 120 soft tissue sarcoma (46%); 71 GIST (27%); 20 desmoid fibromatosis (8%); 4 prefer not to say 4 (1%); 18 missing

Results research priorities

- 1175 different questions came up
- 22 main themes and 21 subthemes



Theme 1: Awareness

25 questions:

- How to create more awareness among GPs?
- How to recognize (signs of) sarcomas?
- How can sarcomas be more reliably recognized by pathologists?
- Which symptoms to look for to detect recurrence?
- What is the risk of doctor's delay?

Theme 2: Consequences of treatment

57 questions:

1. What are the short-term / side effects of specific treatments?

2. What are the late effects of specific treatments?

Theme 3: Cause of sarcoma

39 questions:

- What is the cause of (specific) sarcoma?
- Can a desmoid be hereditary?
- Is there a genetic predisposition for sarcoma/GIST?
- Do people with Lynch syndrome have a larger risk of sarcoma?
- Are sarcomas dependent on hormones?
- Does sarcoma incidence vary per region?
- Why do sarcomas often affect young people?
- Can close exposure to a laptop cause the mutation/GIST?

Theme 4: Research/collaboration strategies

1. Clinical trials (14 questions)

- Could more patients enter clinical trials earlier in their disease trajectory?
- Could responsive patients in clinical trials be further studied?
- Is the use of placebo in clinical trials really necessary?
- Is there a need for different trial designs to study rare cancers like sarcomas?
- What are the possibilities of off-label drugs?
- Why is clinical trial continued when the drug is found to be not effective?

2. Collaboration (10 questions)

- Are sarcoma therapies coordinated among doctors worldwide?
- Is there a difference in approach of metastatic GIST between countries?
- Is there an international treatment strategy for desmoid?
- Why don't clinics work together and agree on procedures?

Theme 5: Communication

40 questions:

- How to optimize information provision to patients to optimize shared decision making?
- How to provide information in an easy to understand way (different topics)?
- What would be the benefits if doctors would respond more to questions/fears of the patient?
- How to talk about the end of life (patient/doctor)?
- How could sarcoma patients be taken more seriously by doctors?

Theme 6: Complementary medicine

16 questions:

- Are there alternative therapy options to reduce risk of recurrence?
- Can complementary / alternative treatments support QoL?
- Can vitamins improve fatigue?
- Is there a role for hypnosis/meditation in follow-up support?
- Which complementary therapy is useful?
- Why is there so little research on complementary medicine/cannabis use?
- Which natural/alternative remedies are recommended to reduce side effects of the therapy?

Theme 7: Diagnosis

1. Diagnostic tests (46 questions)

- What is the optimal imaging method for diagnosis?
- Could full-body ultrasound be an alternative for Xray or CT-scan to detect sarcomas?
- Are there ways other than scans with radiation to detect sarcomas?
- How can sarcomas be more reliably classified by pathologists?
- How to make low-grade sarcomas visible on imaging?
- Is genetic typing comprehensively and early enough involved in GIST?
- Is ultrasound or CT better to detect GIST metastases in follow-up?
- Does each tumor (metastatic GIST) need to be biopsied for mutation analysis?
- What are adequate screening intervals for people with germline mutation and risk of GIST?

2. Diagnostic trajectory (32 questions)

- does earlier recognition lead to improved survival?
- how can sarcoma be diagnosed more quickly?
- how long is sarcoma in the body before diagnosis?
- What is the reason for delay in determination of subtype?
- What is the incidence of incorrect grading of sarcoma?

Theme 8: End of life

71 questions:

- Can chemotherapy treatment accelerate end of life?
- Do patients wish to discuss the end of life?
- How can pain be avoided or alleviated in end of life phase?
- How can QoL be maintained in end of life phase?
- How can you make the end of life phase as pleasant as possible for the patient?
- How do parents experience the last days with their children?
- How good are end of life consultations on weighing opportunities and limitations on QoL?
- Is there variation in end of life care services per country or region?
- What is the perception of patients on how they would like to live their end of life stage?
- What role do relatives/family members play in end of life decisions?

Theme 9: Expert care

61 questions:

- What is the effect of treatment in an expert center?
- Why are there only few experienced doctors in expert centers?
- Which country delivers the best sarcoma care?

Multidisciplinary care (5 questions)

Theme 10: Family/caregivers

15 questions:

- How to support family members?
- How to support parents to support their child?
- What is the role of the caregiver in decision-making?
- What is the role of the caregiver in navigation of care?

Theme 11: Follow-up

43 questions:

- Can follow-up be stratified based on prognostic factors?
- Can GIST be checked with gastroscopy instead of CT scans?
- What is the effect of long term exposure to scans?
- Is it better to use chest X-ray or CT scan for follow-up scans?
- How to deal with scan anxiety?
- How to 'live' in between scans?
- How to follow-up when imatinib is discontinued (in stable patients)?
- What is the optimal duration of follow-up?
- What level of follow-up (burden) is needed burden in case of untreatable disease?

Theme 12: Information

215 questions:

- Where to find information about.....?
- What is the potential benefit of information on how to deal with problems regarding the disease or therapy?

Most often indicating a need for information

Theme 13: Lifestyle

31 questions:

- Do patients need to change their lifestyle?
- How can a sarcoma patient optimize lifestyle to enhance treatment effects?
- What lifestyle interventions could help to better deal with the disease?

1. Nutrition (28 questions)

- Do patients on TKIs need to take supplements?
- Can optimization of diet help to enhance treatment efficacy?
- How can nutrition help for survival/recurrence?
- Can nutrition lower the inflammatory response?

2. Exercise (14 questions)

- Can exercise prevent recurrence/improve survival?
- Can exercise support treatment?
- What is the relationship between exercise and disease progression?
- Does exercise positively influence QoL?
- What is the effect of endurance sports during/after sarcoma?

Theme 14: Peer support

15 questions:

- Where can I find other sarcoma patients?

Most often indicating a need for peer support

Theme 15: Support

64 questions:

- How to provide 'holistic' support and not just focus on the sarcoma treatment?
- What support do patients need in daily life?

1. Physical rehabilitation (7 questions)

- How to physically recover from sarcoma surgery?

2. Psychological support (43 questions)

- Can psychological support improve the disease outcome?
- Can stress management prevent recurrence?
- Does immediate counselling upon terminal diagnosis have a positive effect on survival?
- How can systematic psychological support improve life after treatment?
- How can systematic psychological support improve resilience?
- How to identify yourself in stable conditions? as a cancer patient or not?

3. Practical support (13 questions)

- How can patients be helped to guarantee access to employment?
- How to help patients to benefit from health insurance?
- How many patients return to full time employment?
- How to manage symptoms while working full time?
- How to communicate with employers about the difficulties faced by patients?
- How are patients' earnings affected by sarcoma?

Theme 16: Prevention

10 questions:

- Can sarcoma be prevented?
- How to prevent recurrence?
- Can the incidence of sarcoma be reduced?
- Can lifelong targeted therapy prevent GIST in people with germline mutation?
- Can people influence their risk of getting sarcoma?

Theme 17: Quality of life

45 questions:

- How can sarcoma QoL (during treatment) be improved?
- What is the effect of sarcoma on QoL?
- What is the effect of treatment on QoL?

Theme 18: Risk prediction

32 questions:

- Are there factors to predict the development of metastases at time of diagnosis?
- Are there predictors of which symptoms a patient will experience?
- Can the duration of response to treatment be predicted?
- What are predictors of (prolonged) response to imatinib in GIST?
- What are predictors of long-term stability upon chemotherapy treatment?
- Can the risk of getting sarcoma be predicted?
- Which gene mutations are relevant for a longer survival?
- Do sarcoma/GIST patients risk development of other cancers?
- What is the risk of getting a second sarcoma?

1. Prognosis / survival (48 questions)

- What are indicators of patients' prognosis?
- What can patients do to improve their prognosis?
- Does adjuvant treatment (localized sarcomas) influence survival?
- How many people survive multiple sarcomas?
- What do sarcoma survivors have in common?

2. Recurrence (38 questions)

- How to assess the risk of recurrence/relapse?
- What is the time interval from GIST resection to recurrence without imatinib vs. imatinib?
- How to prevent recurrence?
- What is desmoid recurrence rate?
- What is the role of resection margins in risk of recurrence?
- Which symptoms to look for to detect recurrence?

Theme 19: Sarcoma & subtypes

39 questions:

- Is sarcoma/GIST research lagging behind compared to more common cancers?
- What are specific features of sarcoma?
- What distinguishes sarcomas from other cancers?

Most often indicating a need for information about the disease

Theme 20: Symptoms/problems

96 questions:

- What kind of symptoms can be expected?
- How can you adapt to changes in your body?
- How do patients learn to live with their disease?
- How to deal with suffering from an 'invisible' disease?

1. Fear of recurrence (3 questions)

- How can the fear of recurrence be taken away from patients?

2. Pain (21 questions)

- Does pain medication interfere with the disease?
- How can pain be avoided or alleviated in end of life phase?
- How can post-treatment pain be avoided to limit chance of chronic pain?
- What are the options for pain relief?

3. Mental stress (47 questions)

- How to manage emotions?
- What are the mental effects of sarcoma diagnosis on survivors?
- Is there a relationship between biochemical aspects of the cancer and psychological impact?

Theme 21: Treatment

195 questions:

- Are therapy options for common cancers transferable to sarcoma?
- How to guide treatment decisions based on PROs rather than RECIST criteria?
- What are the risks and benefits of specific treatments?
- Can imatinib treatment of high risk patients be shortened or interrupted?
- Can immunotherapy be offered before chemotherapy?
- Can local treatments work synergistically with systemic treatments in metastatic disease?
- Can pazopanib dosage be personalized/precized to avoid over- or undermedication?
- Can surgical margins be determined during surgery?
- Could combination treatment help to overcome treatment resistance?
- Could existing and new drugs for GIST be given in rotation to prevent resistance to first-line imatinib?
- Does the time of drug intake influence the experience?
- What is the effect of treatment sequence on overall performance?
- How to give patients as much control as possible in treatment decisions?
- What is the influence of environment and lifestyle on effectiveness of treatment?
- Is there an interaction of imatinib with foods (before or after meal intake)?
- Is there a benefit for repurposing of non-oncology drugs for sarcoma treatment?
- What are biomarkers for response to immunotherapy?

Theme 22: Miscellaneous

11 questions:

- How do you create the conditions for a more competent and thinking patient?
- Why are the interests of the pharmaceutical industry placed above the interests of the people?

Results

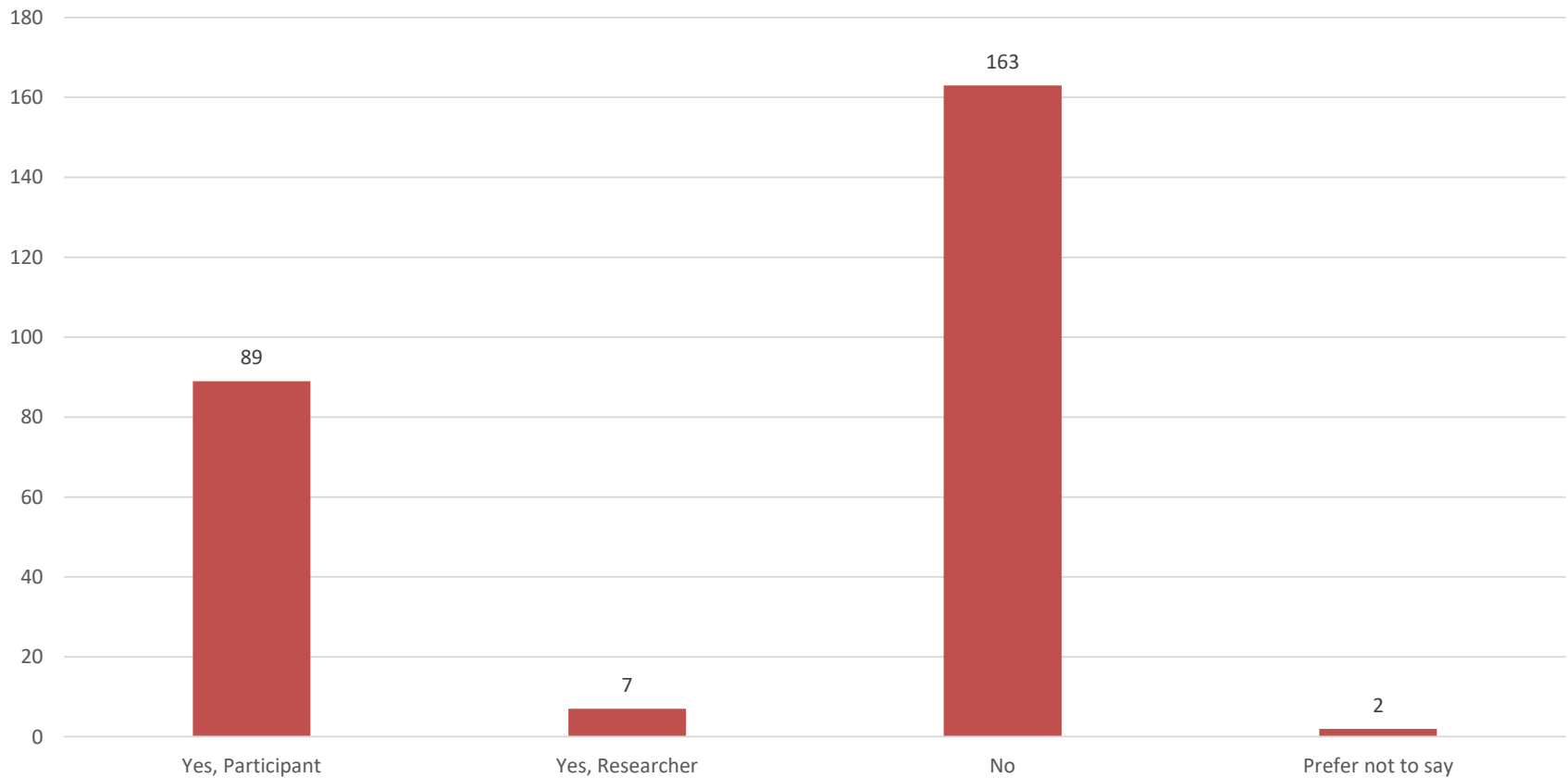
- Health care professionals & researchers
(N=48)

Preliminary: congruence patients/carers and health care professionals?

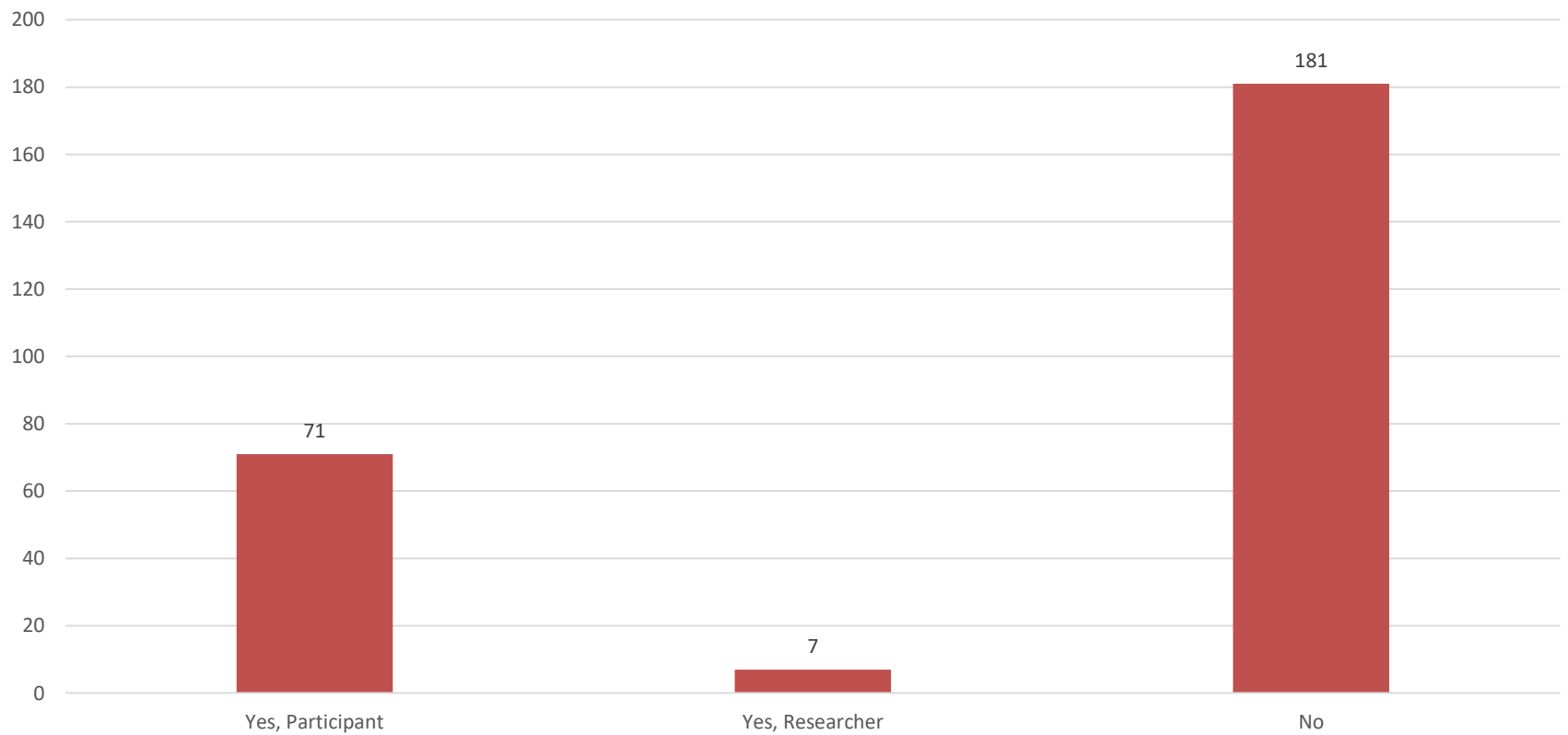
	Patient / carer	Health care professional
Awareness	25	4
Consequences of treatment	57	37
Cause of sarcoma	39	6
Research/collaboration strategies	24	22
Communication	40	3
Complementary medicine	16	1
Diagnosis	78	29
End of life	71	31
Expert care	66	30
Family/caregivers	15	4
Follow-up	43	11
Information	215	11
Lifestyle	73	9
Peer support	15	1
Support	127	21
Prevention	10	1
Quality of life	45	21
Risk prediction	118	32
Sarcoma & subtypes	39	4
Symptoms/problems	167	8
Treatment	195	81
Miscellaneous	11	6

Results patient involvement

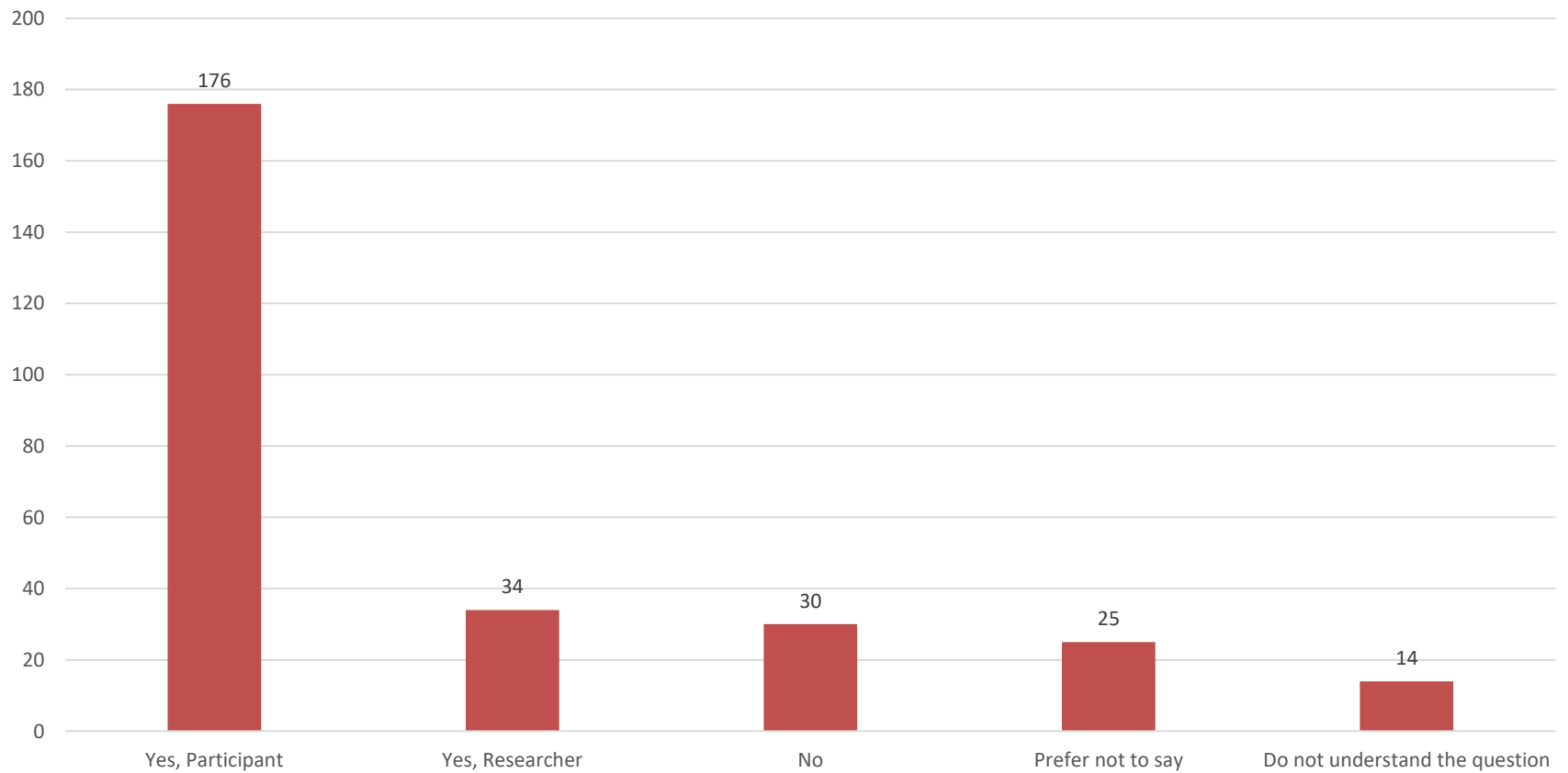
Have you ever been asked to take part in sarcoma research?



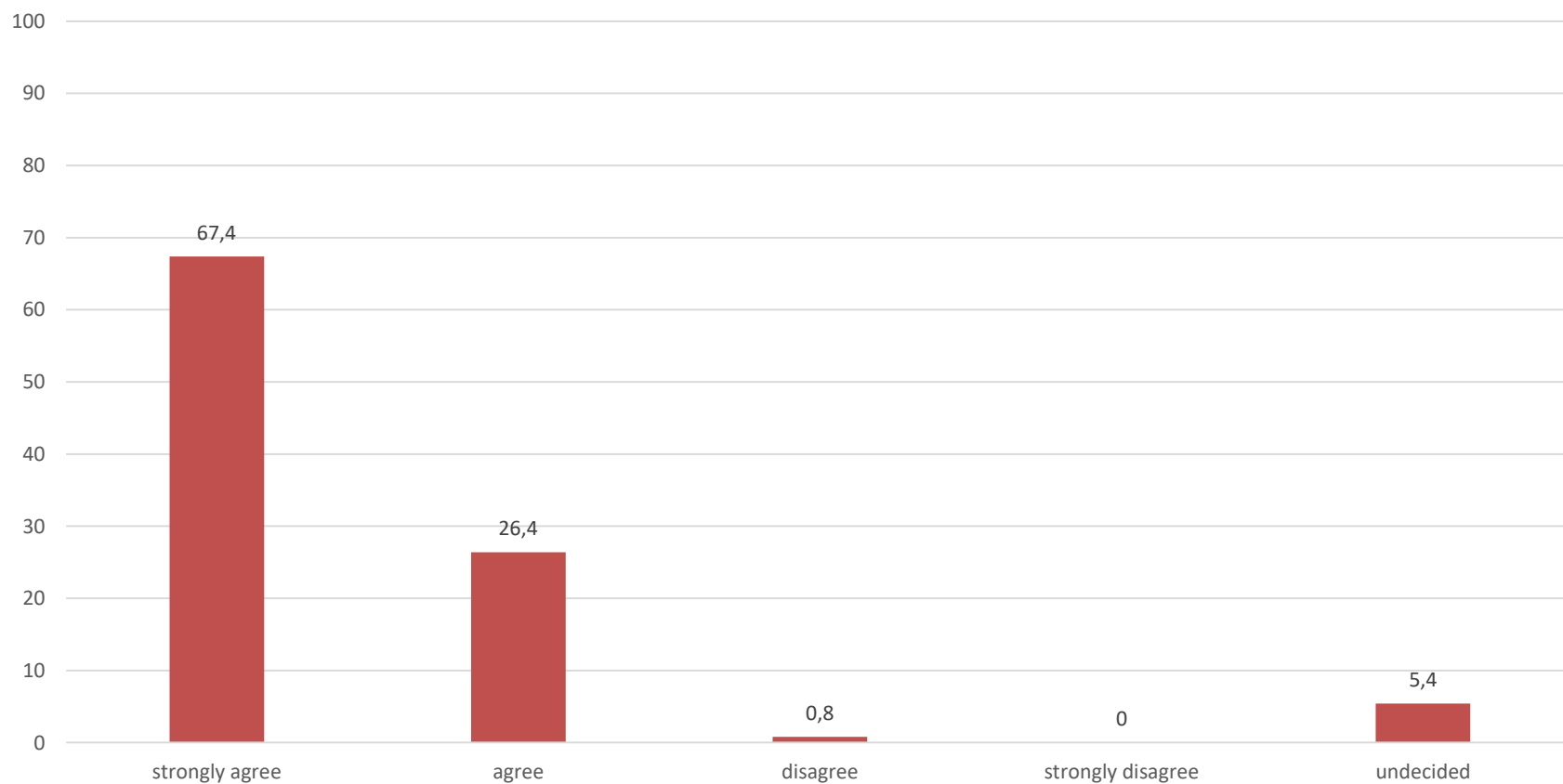
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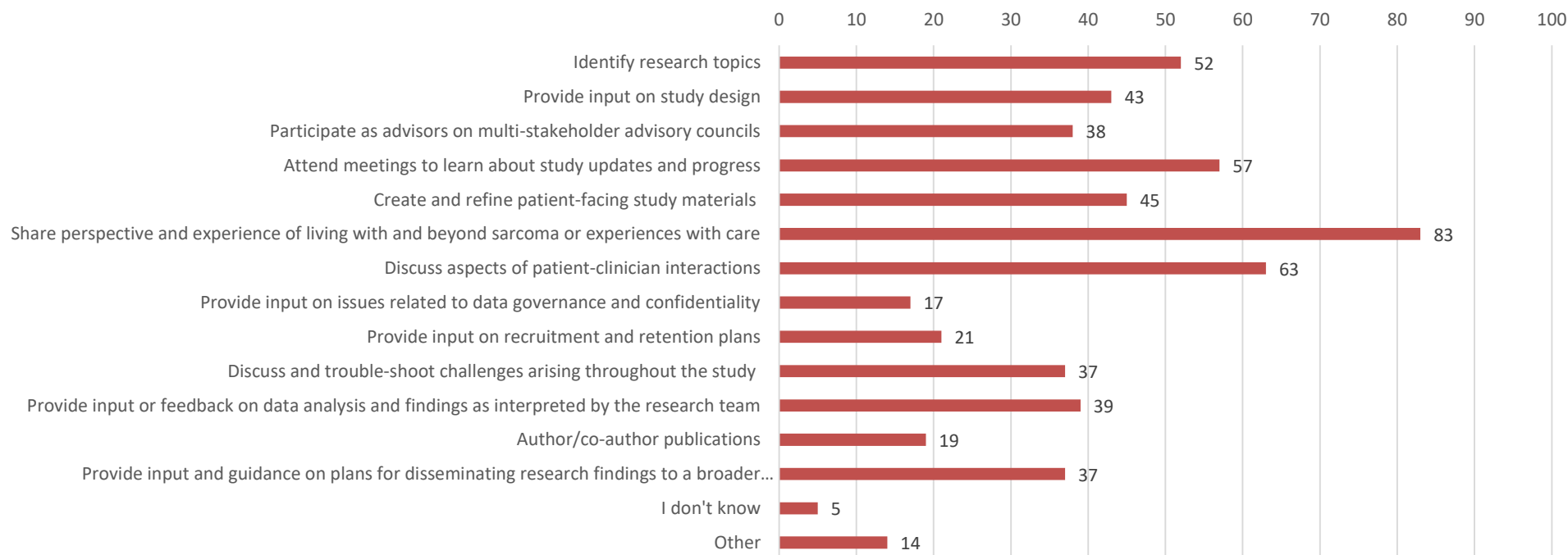
Do you want to be involved in research?



Patients/carers can contribute meaningfully to the research process

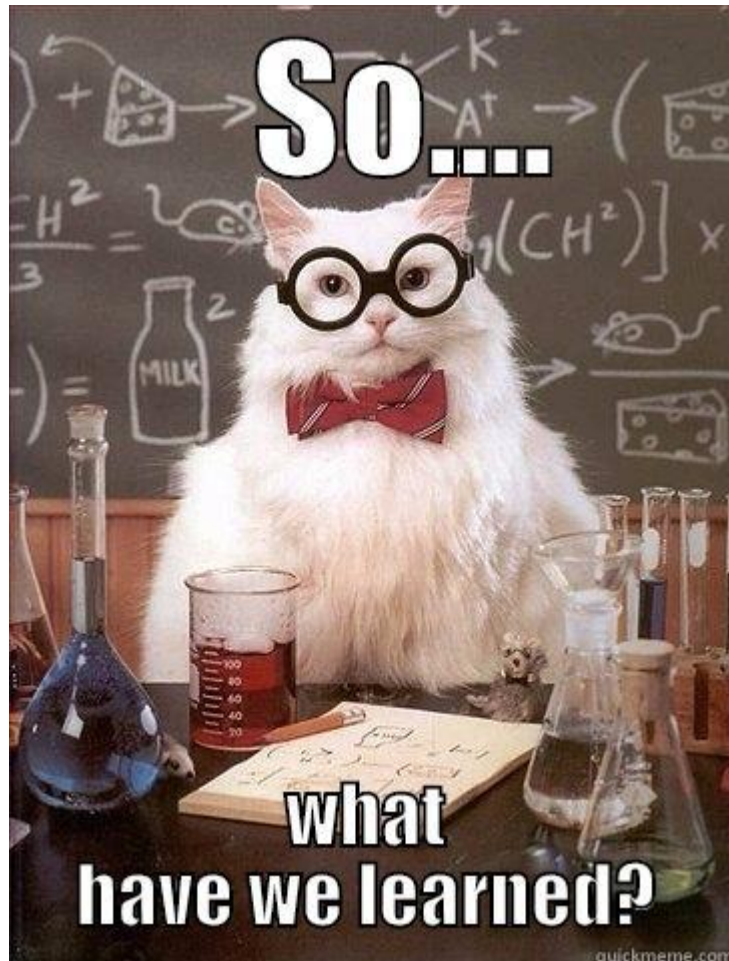


How do you think patients/carers can contribute to the research process?



To be analysed (open questions):

- Where do you think patients/carers can make an impact?
- Why do you think patients/carers do or don't take part in research?
- What could help patients/carers to become involved?



- Quite some questions can be answered by better information provision!
Needs vs. research questions
- Cancer generic vs. sarcoma specific vs. sarcoma subtype specific
- Some of our questions were still difficult to interpret for patients/carers

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Phase 2

- **Step 4: Refining questions/uncertainties and ways to involve patients and carers in research**
- Step 5: Prioritisation

- Compare to available scientific literature
- Create phase 2 questionnaire

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- **Step 5: Prioritisation**

Scientific publication

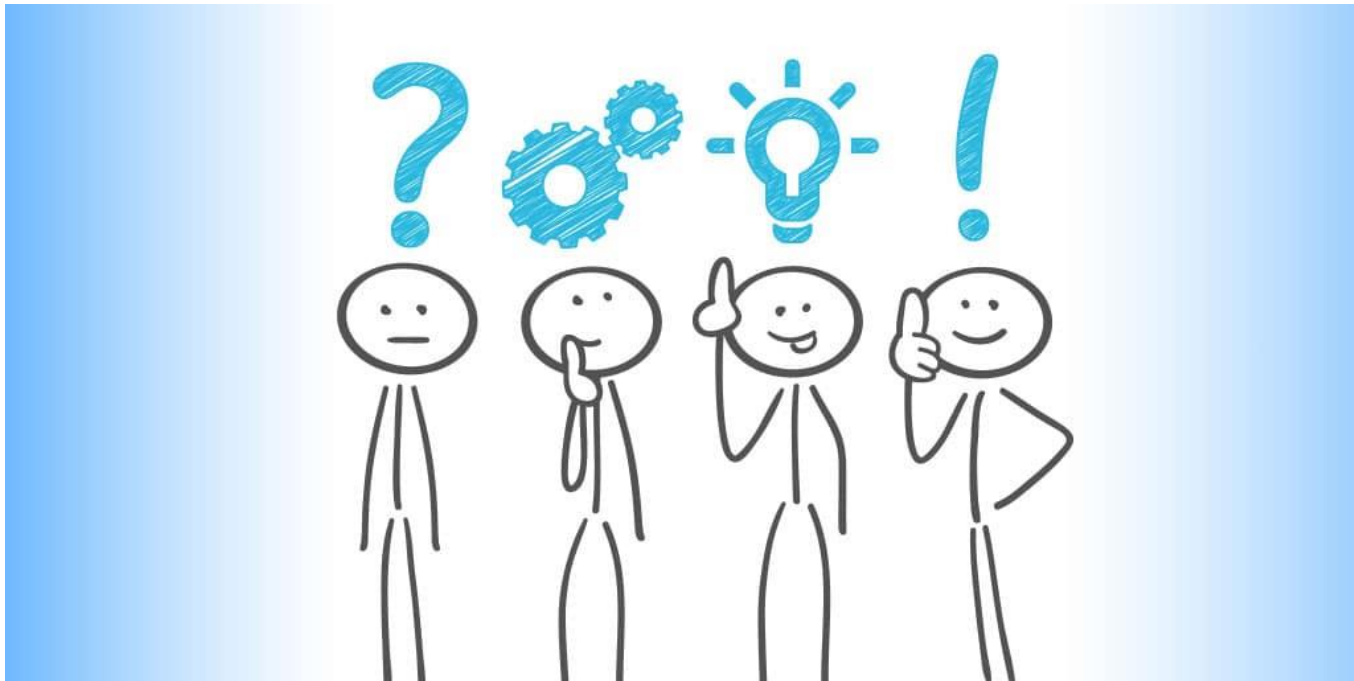


Hopefully the start of a sarcoma patient powered research network!

Big thank you to:

- All participants of the survey
- All steering group members and the SPAEN Board of Directors
- All local patient advocates involved
- Marije Weidema and Milou Reuvers, Kathrin Schuster and Michi Geissler

Questions



Contact: o.husson@nki.nl