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Patient Advocacy
Global Network

EMA-EORTC Workshop
May 24 2024

What is important for patients in addition to RECIST and overall survival

Gerard van Oortmerssen

Co-chair Sarcoma Patients Global Network

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SPAGN’s Global Community 2023

~ 60 Member Groups from all Parts of the World

The Liddy Shriver Sarcoma Initiative – a global initiative for everyone affected by sarcomas

SARCOMA STOWARZYSZENIE POMOCY CHORYM NA MIĘSAKI

swiss sarcoma – VON BETROFFENEN FÜR BETROFFENE

SarcomaUK – The bone & soft tissue cancer charity

TRUST PAOLA GONZATO Rete Sarcoma ONLUS

Sarcoma SFA Foundation of America – Finding the cure in our time

TOGETHER FIGHTING SARCOMA

gençbirder

Info Sarcomes

HENZO KENYA

Friends of Max – Together we share and learn

DESMOID FOUNDATION

SARCOMA COALITION

GIST

gist cancer uk

opv – Associazione Orchestra per la vita ONLUS

GIST SUPPORT ÖSTERREICH – Verein zur Unterstützung von Betroffenen

BONE CANCER RESEARCH TRUST – UNTIL THERE'S A CURE

CUM CURA met zorg

AEAS – Asociación Española de Afectados por Sarcomas

Gyógyulj Velünk Egyesület

SARKOMFÖRENINGEN

CHONDROSARCOMA FOUNDATION

SARKOME.DEUTSCHE SARKOM STIFTUNG

NLMSF

DTRF – THE DESMOID TUMOR RESEARCH FOUNDATION

Sarkomer

GIST.ch – GIST-GRUPPE SCHWEIZ – GROUPE GIST SUISSE

V Care – Support and Hope for those touched by cancer

SOS DESMOÏDE

Patiëntenplatform Sarcomen

CAMPAIGNING FOR CANCER

gsi – GIST Support International

Alianza GIST

CHORDOMA FOUNDATION

The Life Raft GROUP

RARE CANCER 9EHE CHARITY (UK)

Fundación Mari Paz Jiménez Casado – Unidos frente al cáncer

A.I.G. – Associazione Italiana GIST ONLUS

Sock it to Sarcoma!

Suomen Syöpäpotilaat - Cancerpatienterna i Finland ry

GIST Organization

SMARCB1 e.V.

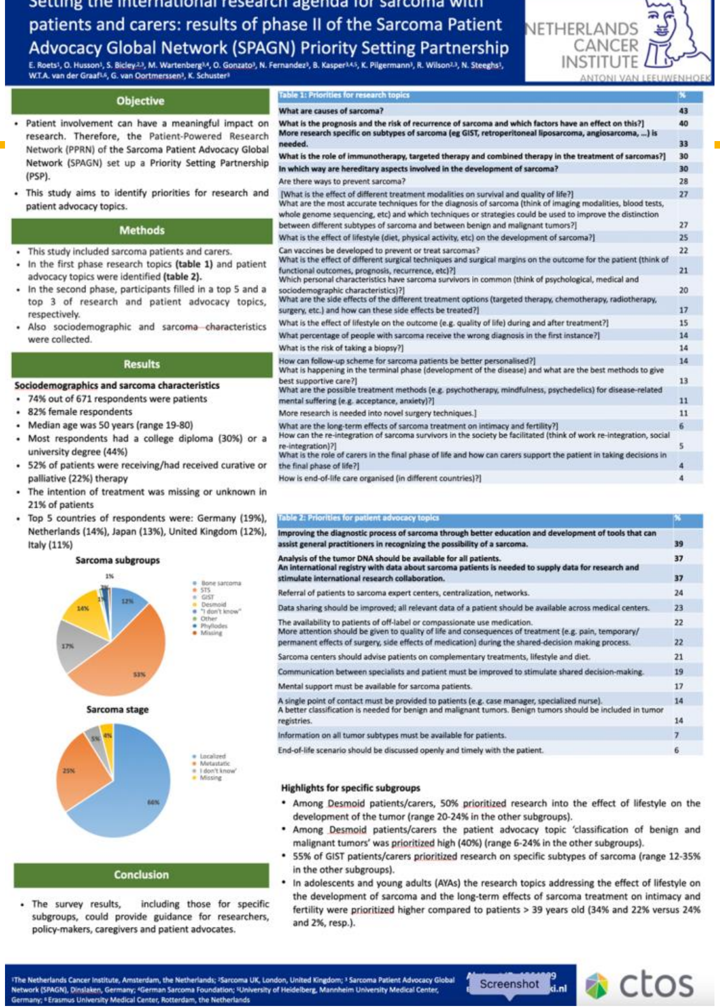
sarcoma alliance

PAWS - GIST – Paediatric Adolescent Wild-type GIST Syndrome – GIST cancer in young people

GIST & STS Alliance for patients - Bulgaria

MIB AGENTS

ASARGA



Setting the international research agenda for sarcoma with patients and carers: results of phase II of the Sarcoma Patient Advocacy Global Network (SPAGN) Priority Setting Partnership

E. Roets[1], O. Husson[1], S. Bicley[2,3], M. Wartenberg[3,4], O. Gonzato[3], N. Fernandez[3], B. Kasper[3,4,5], K. Pilgermann[3], R. Wilson[2,3], N. Steeghs[1], W.T.A. van der Graaf[1,6], G. van Oortmerssen[3], K. Schuster[3]

NETHERLANDS CANCER INSTITUTE ANTONI VAN LEEUWENHOEK

Objective

- Patient involvement can have a meaningful impact on research. Therefore, the Patient-Powered Research Network (PPRN) of the Sarcoma Patient Advocacy Global Network (SPAGN) set up a Priority Setting Partnership (PSP).
- This study aims to identify priorities for research and patient advocacy topics.

Methods

- This study included sarcoma patients and carers.
- In the first phase research topics **(table 1)** and patient advocacy topics were identified **(table 2).**
- In the second phase, participants filled in a top 5 and a top 3 of research and patient advocacy topics, respectively.
- Also sociodemographic and sarcoma characteristics were collected.

Results

Sociodemographics and sarcoma characteristics

- 74% out of 671 respondents were patients
- 82% female respondents
- Median age was 50 years (range 19-80)
- Most respondents had a college diploma (30%) or a university degree (44%)
- 52% of patients were receiving/had received curative or palliative (22%) therapy
- The intention of treatment was missing or unknown in 21% of patients
- Top 5 countries of respondents were: Germany (19%), Netherlands (14%), Japan (13%), United Kingdom (12%), Italy (11%)

Conclusion

- The survey results, including those for specific subgroups, could provide guidance for researchers, policy-makers, caregivers and patient advocates.

Table 1: Priorities for research topics	%
What are causes of sarcoma?	**43**
What is the prognosis and the risk of recurrence of sarcoma and which factors have an effect on this?]	**40**
More research specific on subtypes of sarcoma (eg GIST, retroperitoneal liposarcoma, angiosarcoma, …) is needed.	**33**
What is the role of immunotherapy, targeted therapy and combined therapy in the treatment of sarcomas?]	**30**
In which way are hereditary aspects involved in the development of sarcoma?	**30**
Are there ways to prevent sarcoma?	28
[What is the effect of different treatment modalities on survival and quality of life?]	27
What are the most accurate techniques for the diagnosis of sarcoma (think of imaging modalities, blood tests, whole genome sequencing, etc) and which techniques or strategies could be used to improve the distinction between different subtypes of sarcoma and between benign and malignant tumors?]	27
What is the effect of lifestyle (diet, physical activity, etc) on the development of sarcoma?]	25
Can vaccines be developed to prevent or treat sarcomas?	22
What is the effect of different surgical techniques and surgical margins on the outcome for the patient (think of functional outcomes, prognosis, recurrence, etc)?]	21
Which personal characteristics have sarcoma survivors in common (think of psychological, medical and sociodemographic characteristics)?]	20
What are the side effects of the different treatment options (targeted therapy, chemotherapy, radiotherapy, surgery, etc.) and how can these side effects be treated?]	17
What is the effect of lifestyle on the outcome (e.g. quality of life) during and after treatment?]	15
What percentage of people with sarcoma receive the wrong diagnosis in the first instance?]	14
What is the risk of taking a biopsy?]	14
How can follow-up scheme for sarcoma patients be better personalised?]	14
What is happening in the terminal phase (development of the disease) and what are the best methods to give best supportive care?]	13
What are the possible treatment methods (e.g. psychotherapy, mindfulness, psychedelics) for disease-related mental suffering (e.g. acceptance, anxiety)?]	11
More research is needed into novel surgery techniques.]	11
What are the long-term effects of sarcoma treatment on intimacy and fertility?]	6
How can the re-integration of sarcoma survivors in the society be facilitated (think of work re-integration, social re-integration)?]	5
What is the role of carers in the final phase of life and how can carers support the patient in taking decisions in the final phase of life?]	4
How is end-of-life care organised (in different countries)?]	4

Table 2: Priorities for patient advocacy topics	%
Improving the diagnostic process of sarcoma through better education and development of tools that can assist general practitioners in recognizing the possibility of a sarcoma.	**39**
Analysis of the tumor DNA should be available for all patients.	**37**
An international registry with data about sarcoma patients is needed to supply data for research and stimulate international research collaboration.	**37**
Referral of patients to sarcoma expert centers, centralization, networks.	24
Data sharing should be improved; all relevant data of a patient should be available across medical centers.	23
The availability to patients of off-label or compassionate use medication.	22
More attention should be given to quality of life and consequences of treatment (e.g. pain, temporary/ permanent effects of surgery, side effects of medication) during the shared-decision making process.	22
Sarcoma centers should advise patients on complementary treatments, lifestyle and diet.	21
Communication between specialists and patient must be improved to stimulate shared decision-making.	19
Mental support must be available for sarcoma patients.	17
A single point of contact must be provided to patients (e.g. case manager, specialized nurse).	14
A better classification is needed for benign and malignant tumors. Benign tumors should be included in tumor registries.	14
Information on all tumor subtypes must be available for patients.	7
End-of-life scenario should be discussed openly and timely with the patient.	6

Highlights for specific subgroups

- Among Desmoid patients/carers, 50% prioritized research into the effect of lifestyle on the development of the tumor (range 20-24% in the other subgroups).
- Among Desmoid patients/carers the patient advocacy topic 'classification of benign and malignant tumors' was prioritized high (40%) (range 6-24% in the other subgroups).
- 55% of GIST patients/carers prioritized research on specific subtypes of sarcoma (range 12-35% in the other subgroups).
- In adolescents and young adults (AYAs) the research topics addressing the effect of lifestyle on the development of sarcoma and the long-term effects of sarcoma treatment on intimacy and fertility were prioritized higher compared to patients > 39 years old (34% and 22% versus 24% and 2%, resp.).

[1]The Netherlands Cancer Institute, Amsterdam, the Netherlands; [2]Sarcoma UK, London, United Kingdom; [3] Sarcoma Patient Advocacy Global Network (SPAGN), Dinslaken, Germany; [4]German Sarcoma Foundation; [5]University of Heidelberg, Mannheim University Medical Center, Germany; [6] Erasmus University Medical Center, Rotterdam, the Netherlands

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Wishes of patients for research and advocacy:

- More research especially on different subtypes
- More emphasis on quality of life

Rare/Ultra rare subtypes

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For many patients a trial is their last hope

- Need for (new) treatments and new methods
- Baskets, Repurposing
- Other criteria than RECIST and overall survival
- Quality of life

Patient Reported Outcomes

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- PROs in addition to clinical data
- Symptomatic side effects
- Overall side effect impact
- Physical function
- Social function

Patient Reported Outcomes

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- ‘Hidden’ side effects
- Some AEs challenging to observe (fatigue)
- Absence of patient perspective may lead to underestimation
- Develop supportive measures

Digital revolution

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Use of smartphones and Internet for patient reporting opens up opportunities for longitudinal data collection

Empowerment of patients

Patients take action together

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- Set up international registry
- Tissue bank
- Discussion forum / social media used to share experiences

Exaples: GIST, Chordoma, EHE,..

Collaboration with researchers and specialists

Example

Facebook Group

6000 memnbers

>100,000 posts

GIST Support International (GSI) is an all-volunteer organization that provides education and support for GIST patients and their famili

GIST Support International

Info

GIST Support International (GSI) is an all-volunteer organization that provides education and support for GIST patients and their families.

Schrijf iets...

Comparison: FB with trial (avapritinib)

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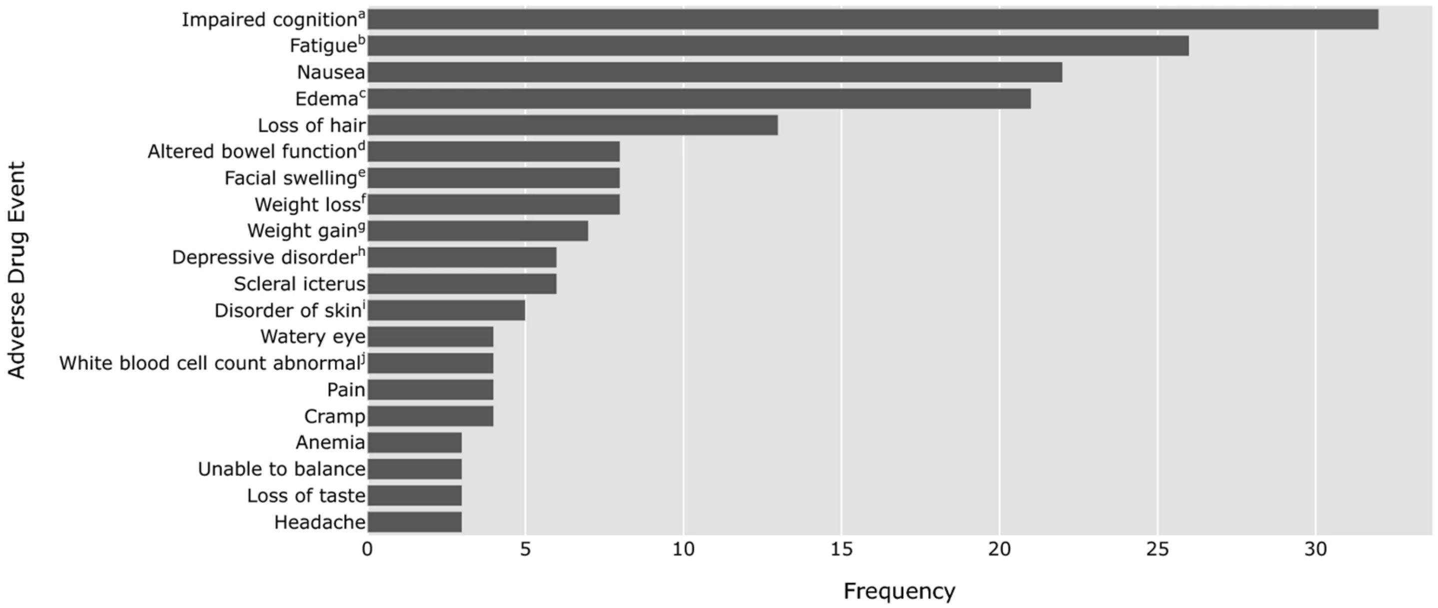

[a] includes Amnesia
[b] includes Drowsy, Tired, Exhaustion, Asthenia, Lethargy and Lack of energy
[c] includes Body fluid retention and Periorbital edema
[d] includes Diarrhea and Constipation
[e] includes Swelling of eyelid and Swelling of structure of eye
[f] includes Weight decreased
[g] includes Excessive weight gain
[h] includes Mild depression and Major depressive disorder
[i] includes Pruritus of skin and Eruption
[j] includes Leukopenia

Grading: comparison with trial

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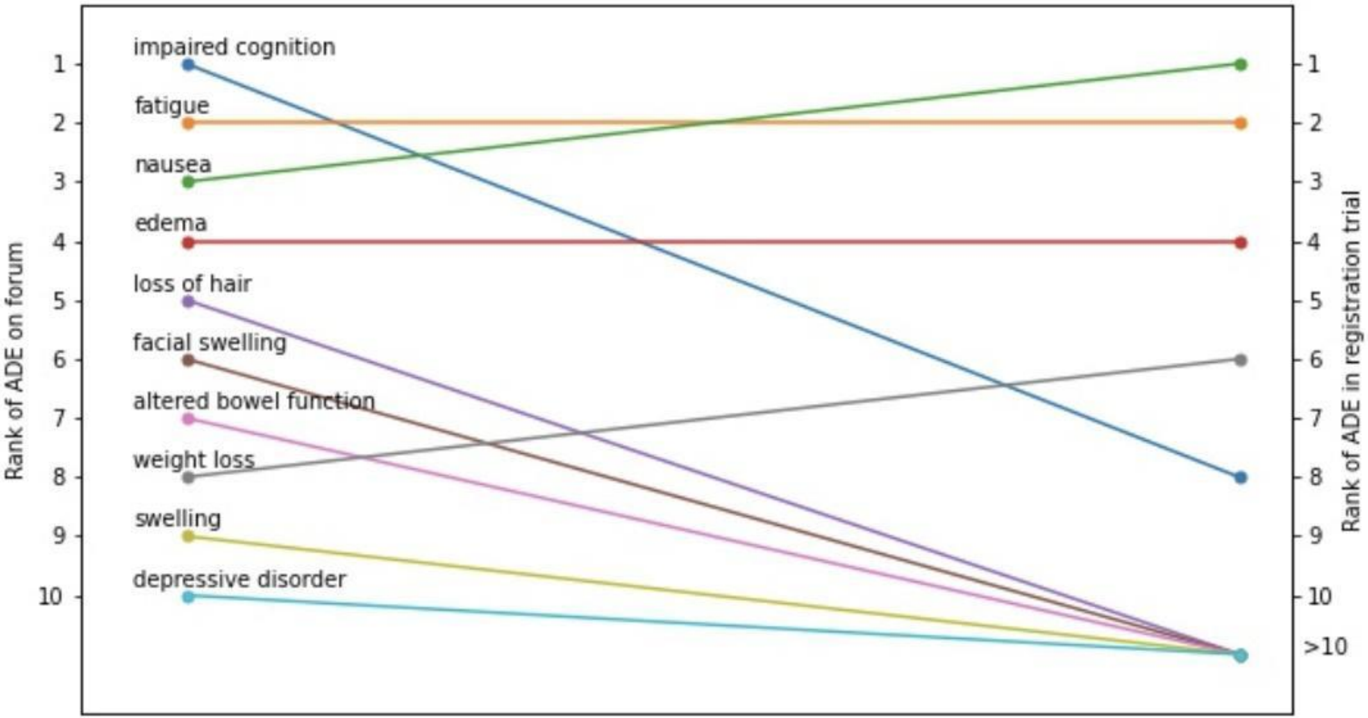

Side effects as obtained from Facebook discussions compared to official trial results

Comparison with survey

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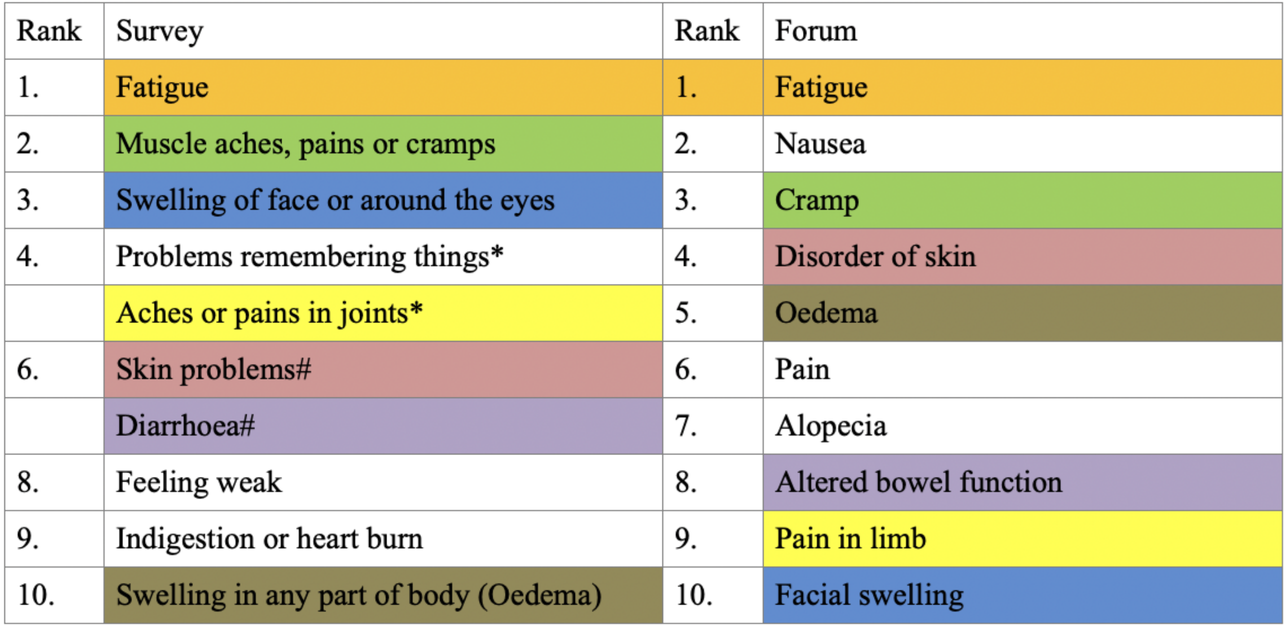
Table 4: Ranking of prevalence of symptoms related to imatinib in survey study and forum study



Rank	Survey	Rank	Forum
1.	Fatigue	1.	Fatigue
2.	Muscle aches, pains or cramps	2.	Nausea
3.	Swelling of face or around the eyes	3.	Cramp
4.	Problems remembering things*	4.	Disorder of skin
	Aches or pains in joints*	5.	Oedema
6.	Skin problems#	6.	Pain
	Diarrhoea#	7.	Alopecia
8.	Feeling weak	8.	Altered bowel function
9.	Indigestion or heart burn	9.	Pain in limb
10.	Swelling in any part of body (Oedema)	10.	Facial swelling

*same prevalence(52%) # same prevalence (50%)

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Conclusions

- Clinical trials are important for patients with (ultra) rare sarcomas
- Important to look at other criteria than RECIST
- Patient Reported Outcomes have to play an important role
- Collaboration between patients/clinicians/researchers is important