Poster 5700

The Development and Evaluation of a Patient and Carer Guide for Selumetinib Treatment in Neurofibromatosis Type 1 in the UK

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STUDY OBJECTIVE

- In this study, a comprehensive patient and carer guide was developed to aid navigation of the selumetinib treatment journey in NF1
  - The guide was distributed across two national NF1 treatment centres in the UK to enable patient and carer feedback to be collected on content and usability

CONCLUSIONS

- The patient and carer guide for selumetinib treatment has proven to be a valuable resource by enhancing understanding and management of the treatment journey
- All approached families were engaged in the survey and wanted to provide their feedback on the patient and carer guide for selumetinib treatment in NF1
  - Feedback indicates that the content is set out in a user-friendly format, is easy to navigate and provides reassurance to patients and carers regarding the treatment journey
  - Participants noted that the patient and carer guide for selumetinib treatment in NF1 was a useful reference for a clinical setting, at home, at school, and with family
- As a result of the evaluations conducted in this study, the patient and carer guide for selumetinib treatment will be improved by incorporating the suggestions made by the participating families
- Future developments will explore the feasibility of converting the guide into a mobile application, thereby providing an accessible and interactive platform

PLAIN LANGUAGE SUMMARY

Why did we perform this research?
This research aimed to create a helpful guide for patients and their carers to navigate the treatment journey with selumetinib. The goal was to support families and improve communication between them and healthcare providers.

How did we perform this research?
Clinical nurse specialists from two National NF1 treatment centres in the UK developed a comprehensive guide to aid navigation of the selumetinib treatment journey. The guide included picture guidance and coloured tabs to cover various aspects of the treatment journey. Patients and carers provided feedback on the guide via questionnaires. The feedback was collected over three months and used a scale of 1 to 5 to rate usefulness, ease of use, ability of the guide to help with understanding of the treatment journey, and ability of the guide to provide reassurance.

What were the findings of this research and what are the implications?
The study found that patients and carers rated the guide highly for its user-friendliness and ease of navigation. Most participants found it helpful at home and felt reassured by the information provided. The sections on daily care routines and managing side effects were particularly useful. Some recommended adding a section on mental health and providing more space for appointments. The findings suggest that the guide significantly enhances understanding and management of the selumetinib treatment journey in NF1. It also facilitates better communication with schools and extended family members. Future improvements based on user feedback will make the guide even more effective.

Where can I access more information?
For more information on the selumetinib treatment journey, patients and carers can reach out to their healthcare team.

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Poster

Plain language summary

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BACKGROUND

- Neurofibromatosis type 1 (NF1) is an autosomal dominant genetic condition with a birth incidence of 1 in 2500/3000[1]
  - NF1 can lead to the development of plexiform neurofibromas (PN) in 20–50% of patients[2–5]
- Selumetinib (ARRY-142886, AZD6244), an oral inhibitor of mitogen-activated protein kinase kinases 1 and 2, has been approved in the UK for paediatric patients aged ≥3 years with NF1 and symptomatic, inoperable PN[6]
- Given the complexity of NF1,[7] there is an unmet need for resources to support patients and their families through their treatment journeys and to aid communication between different healthcare providers

METHODS

- The comprehensive patient and carer guide for selumetinib treatment in NF1 was developed by two clinical nurse specialists from each National UK NF1 centre
- Picture guidance was included to support children and patients with learning difficulties
- The guide was split into sections, which were indicated by coloured tabs; the sections covered various aspects of the treatment journey, including how to take selumetinib, daily oral and skin care, possible side effects, a space to record upcoming medical appointments and the contact details for patient support groups (**Figure 1**)
- Patients and carers were asked to complete a hard copy questionnaire either in clinic or at home regarding their experience of using the patient and carer NF1 selumetinib treatment guide
  - Feedback was collected after 3 months of using the guide
- A scale of 1 to 5 was used for feedback (5 being the highest score and 1 being the lowest score) regarding usefulness, ease of use, ability of the guide to aid understanding of the patient journey, and ability of the guide to provide reassurance

Figure 1. Sections included in the patient and carer guide for selumetinib treatment in NF1

NF1, neurofibromatosis type 1.

RESULTS

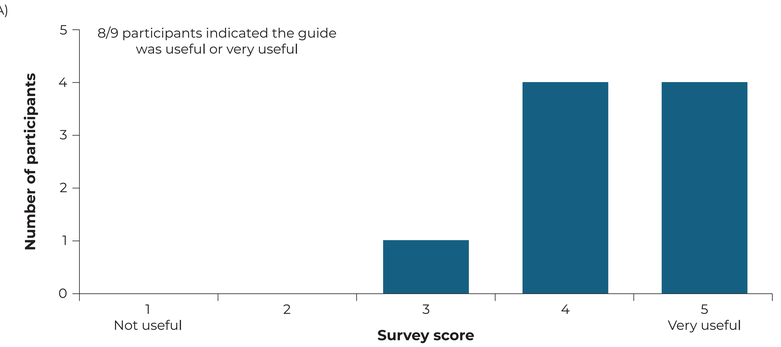
Study population
- A total of nine different patients or carers participated in the study; the age of the patients was not recorded
  - Of these participants, 8/9 had already initiated selumetinib treatment

Responses to the survey

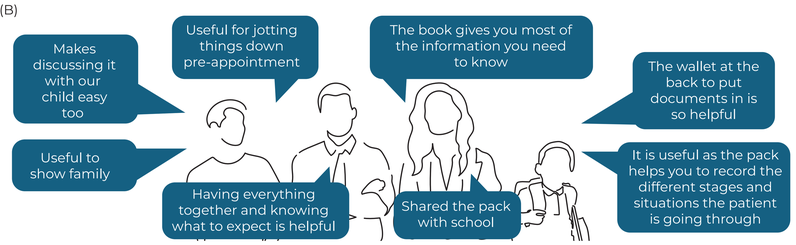
How useful is the patient and carer guide for participants receiving selumetinib treatment in NF1?
- High feedback scores from patients and carers indicated that the guide was useful (**Figure 2**) and user-friendly (**Figure 3**)

Figure 2. (A) Survey scores in response to the question 'How useful is the patient and carer guide for selumetinib treatment in NF1?' and (B) reasons given for survey scores

(A)

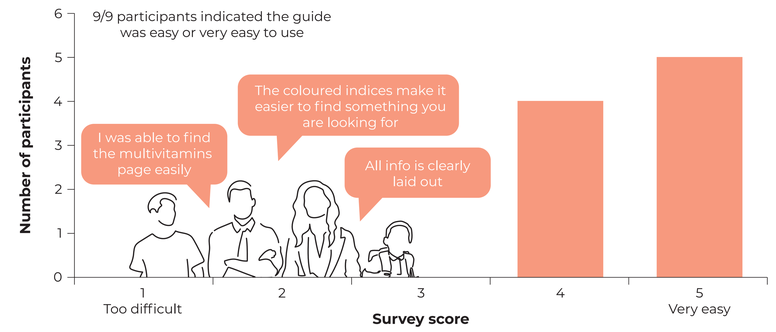


(B)



NF1, neurofibromatosis type 1.

Figure 3. Survey scores and reasons given in response to the question 'How easy to use is the patient and carer guide for selumetinib treatment in NF1?'

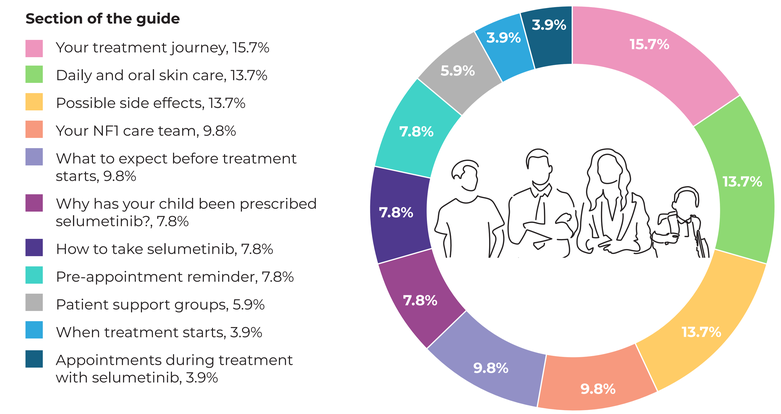


NF1, neurofibromatosis type 1.

- 8/9 participants indicated that the guide is helpful at home, and 1/9 participants indicated that they were unsure if the guide is helpful at home
- In total, 8/9 participants felt they understood what was next in their child's treatment journey (rating either 4/5 or 5/5)
- 8/9 participants felt reassured by the information provided (rating either 4/5 or 5/5), with visual aids enhancing comprehension

Which sections of the patient and carer guide for selumetinib treatment in NF1 are most useful?
- Users found the sections on daily care routines and what to expect during the treatment journey most helpful (**Figure 4**)

Figure 4. Survey responses to the question 'Which sections of the patient and carer guide for selumetinib treatment in NF1 did you find most useful?'



Respondents could select more than one answer.
NF1, neurofibromatosis type 1.

- **Use of the guide has facilitated communication with schools and members of extended family, thereby creating a wider support network for the patient**
- **Additionally, the guide assisted in providing information on possible side effects and keeping track of appointments**
- **One participant valued the importance of the patient and carer treatment guide for NF1 by comparing it to the family-held oncology records used in cancer care**

Is there anything missing from the patient and carer guide for selumetinib treatment in NF1?
- The integration of a section on mental health was recommended so that users can document and track their emotional well-being
- The inclusion of more space for recording appointments was also recommended

When should the patient and carer guide for selumetinib treatment in NF1 be distributed to patients and their carers?
- Carers had different opinions on when they should receive the guide (**Table 1**)

Table 1. Carer responses to the question 'At what point in your child's treatment journey would the patient and carer guide for selumetinib treatment in NF1 be most useful to receive?'

Point in treatment journey	Responses from carers, n
At the NF1 centre, before your child is referred to a paediatric oncologist	**3**
When your child starts selumetinib treatment	**3**
When you have your first appointment with a paediatric oncologist	1
Before your child has their first pre-treatment test	1
When you have an appointment with an oncology nurse	1

NF1, neurofibromatosis type 1.

LIMITATIONS

- Small sample size
- The patient and carer guide was circulated to families of patients who were already receiving treatment with selumetinib (8/9 participants)
  - Future studies are therefore required to obtain feedback on the usefulness of the guide when provided to families before initiation of selumetinib treatment

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Conflicts of interest
Mandy Myers has received financial support for development of the patient and carer guide; consulting fees; payment or honoraria for lectures, presentations, speaker bureaus or educational needs, and travel support for attending meetings from Alexion, AstraZeneca Rare Disease. Carol Irving has received payment or honoraria for lectures, presentations, speaker bureaus or educational needs, and travel support for attending meetings from Alexion, AstraZeneca Rare Disease. Katrina Kettle has received consulting fees and support for attending meetings and/or travel from Alexion, AstraZeneca Rare Disease. Nadira Bullock is an employee of Alexion, AstraZeneca Rare Disease, and holds shares in AstraZeneca. Judith Eelloo has received financial support for development of the patient and carer guide, and consulting fees from Alexion, AstraZeneca Rare Disease. Mandy Myers, Carol Irving, and Katrina Kettle also declare participation on a Data Safety Monitoring Board or Advisory Board for Alexion, AstraZeneca Rare Disease.

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