

Comparison of Early Response to Selumetinib Therapy in Adolescents and Children With NF1-related Plexiform Neurofibromas

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Abstract

Background/Aim: Following approval from the European Medicines Agency, selumetinib has emerged as a targeted therapy for pediatric patients aged ≥ 3 years with inoperable, symptomatic NF1-related plexiform neurofibromas (PNs). In Poland, this treatment has been reimbursed since January 2024 under a national therapeutic program funded

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by the National Health Service. Nevertheless, the efficacy and safety of selumetinib in subgroups within the pediatric population remain unclear. This study aimed to evaluate the efficacy and safety of selumetinib treatment in adolescents (≥ 16 years old) compared with younger children with NF1-related PNs.

Patients and Methods: A total of 111 patients were included in the program over a two-year period. Patients were divided into two groups according to age: adolescents (≥ 16 years of age) and children (< 16 years of age). Demographic characteristics, PN location, treatment efficacy and adverse effects were analyzed and compared between the two groups.

Results: Response rates at the first and second evaluation time point were similar between the groups. However, at the third time point, the response rate was significantly higher in children vs. adolescents (56.0% vs. 18.2%; odds ratio=5.7; 95% confidence interval=1.02-32; $p=0.035$). Skin-related adverse effects were the most common in both groups, and only isolated cases required selumetinib dose reduction.

Conclusion: Early response to selumetinib therapy is achieved faster in children than in adolescents. Selumetinib is generally well tolerated in both age groups.

Keywords: Neurofibromatosis type 1, plexiform neurofibroma, selumetinib, adolescents, children.

Introduction

Neurofibromatosis type 1 (NF1) is a rare genetic disease occurring in 1 out of 3,000 people (1-3). NF1 results from a heterozygous loss-of-function mutation in the *Nf1* gene, which is responsible for regulating the RAS-MAPK signaling pathway essential for growth, proliferation, and differentiation of cells. Somatic loss of heterozygosity in *Nf1* gene gives rise to characteristic clinical features such as café-au-lait macules, bone dysplasia, and tumor formation (3).

Plexiform neurofibroma (PN), one of the most common NF1-dependant tumors, develops in approximately 50-60% of patients. PN, although histologically benign, carries a risk of transformation to malignant peripheral nerve sheath tumor (MPNST) and can cause numerous morbidities depending on its location (including pain and disfigurement) (1-6).

The standard treatment approach for PN is surgical excision. However, complete resection is often unfeasible due to the tumor's anatomical location and the associated risk of complications. Another issue is the high rate of recurrence after the debulking surgery only, observed in approximately 40-50% of patients (3).

In June 2021, selumetinib, MEK1/2 inhibitor, received approval from the European Medicines Agency as a novel

targeted therapy for pediatric patients aged ≥ 3 years with inoperable and symptomatic PNs, representing a major advancement in PN treatment (7). Furthermore, cases of significant clinical improvement after postoperative selumetinib treatment have been reported (8). Selumetinib was also assessed for its potential use in thyroid cancer as MAPK inhibition attenuates SREBP1 expression in *BRAF*-mutant thyroid cancer cells (9).

As of January 2024, selumetinib has been introduced in Poland as a fully reimbursed therapy for pediatric patients with NF1-related PNs meeting the National Health Fund (NHF) eligibility criteria. Although the use of selumetinib for treating NF1-related PNs in pediatric patients has been under significant investigation, its safety and efficacy in adults remain largely unknown (3). The same concerns age-specific information about subgroups within the pediatric population. Nevertheless, studies conducted to date in a population of Polish pediatric patients with PN due to NF1 indicate that the efficacy and safety profile of selumetinib treatment in the Polish population are comparable to those reported in the SPRINT study (10).

The aim of this study was to assess the efficacy and safety of selumetinib treatment in adolescents (≥ 16 years old) and younger children with NF1-related PNs.

Patients and Methods

Study design. The prospective multi-center analysis was performed on a cohort of pediatric patients with NF1-related PNs included in the NHF-reimbursed therapeutic program in Poland.

Patients. From January, 1st, 2024 to July, 31st, 2025, a total number of 111 patients were included into therapeutic program with selumetinib. We divided the cohort into two groups based on patient's age – adolescents (≥ 16 years of age) and children (< 16 years of age).

Analyzed factors. Demographic characteristics, location of PNs, treatment efficacy (due to response criteria) and adverse effects were analyzed and compared between adolescent and children patients.

Tumor response evaluation and response criteria. Pediatric patients with NF-1 related PNs received selumetinib orally twice daily. Imaging analysis [volumetric magnetic resonance imaging (MRI)], laboratory tests and observer-reported outcome measures were performed in every course of the therapy (1 course: 4-6 cycles, 1 cycle: 28 days). Partial response (PR) was defined as a decrease in tumor volume $\geq 20\%$ from baseline, whereas confirmed partial response (CPR) was proved by meeting the above criterion lasting for at least two consecutive MRI periodic scans. Stable disease (SD) was considered as tumor volume changes between -20% to $+20\%$ from baseline. Progressive disease (PD) was defined as an increase of the tumor volume $\geq 20\%$ from the baseline [confirmed progressive disease (CPD)] – proved by meeting the PD criterion in at least two consecutive MRI periodic scans).

Adverse effects assessment. As in the case of tumor response analysis, clinical examination, laboratory tests, ophthalmological and cardiological examinations were performed at baseline and on regular basis at every therapy check point in accordance with the program

requirements. Adverse effects were graded according to the CTCAE scale v. 6.0 (11).

Ethical considerations. The study was performed within the National Health Service therapeutic program. The study was approved by local Bioethical Committee of Collegium Medicum in Bydgoszcz (decision KB 381/2024).

Statistical analysis. Comparison of response between groups was performed with two-tailed χ^2 -test or Fischer exact test and odds ratio (OR) values with 95% confidence interval (CI); p -value < 0.05 was considered statistically significant.

Results

Demographic characteristics. We analyzed prospectively 111 pediatric patients qualified for selumetinib treatment by the end of July 2025. Among the entire group, 66% (73/111) were male and 34% (38/111) were female. Based on age, 29% (32/111) of the patients (22 male and 10 female) were assigned to the adolescents group whereas 71% (79/111) to children group (51 male patients and 28 female patients). In the adolescent group two patients were excluded from the program before the first drug dispensation – one of them due to reaching 18 years of age at the time of treatment initiation and the second one due to withdrawal of parental consent before treatment commenced.

Location of the PN. In adolescents, the most common location was head and neck (46.9%, 15/32), followed by chest (31.3%; 10/32), limb (15.6%; 5/32), abdomen and paravertebral region (each 12.5%, 4/32), pelvis and back (each 3.1%; 1/32). In seven patients (21.9%) clinically significant PNs were located in more than one anatomical region (Table I).

In children, the most common location was head and neck (51.9%; 41/79), followed by chest, abdomen and limb (each 20.3%; 16/79), paravertebral region (7.6%; 6/79) and pelvis (1.3; 1/79). In 12 patients (15.2%) clinically significant PNs were in more than one anatomical region (Table I).

Table I. Plexiform neurofibroma (PN) location in adolescents and children.

PN location	Adolescents (n=32) n (%)	Children (n=79) n (%)
Head and neck	15 (46.9%)	41 (51.9%)
Chest	10 (31.3%)	16 (20.3%)
Limb	5 (15.6%)	16 (20.3%)
Abdomen	4 (12.5%)	16 (20.3%)
Paravertebral region	4 (12.5%)	6 (7.6%)
Pelvis	1 (3.1%)	1 (1.3%)
Back	1 (3.1%)	0 (0.0%)

Efficacy of treatment. Until the end of July 2025, 24 adolescents and 62 children completed the first course of the therapy. Partial regression occurred in 33.5% (8/24) of adolescents and 30.6% (19/62) of children (p =non-significant). Stabilization of the PN’s volume was observed in 62.5% (15/24) of adolescents and 69.4% (43/62) of children. One patient in the adolescent group experienced progression disease (1/24 4.2%), PD was not observed in children (Table II).

Efficacy after two courses of selumetinib therapy could be assessed in 19 adolescents and 39 children. 31.6% (6/19) adolescents and 41.0% (16/39) children experienced PR (p =non-significant). Target lesion (TL) was stable in 57.9% (11/19) of adolescents and 53.8% (21/39) of children. Two adolescents experienced PD (10.5%, 2/19), one of them was excluded from the program, and the other one was conditionally maintained on selumetinib treatment. PD was observed in 2.6% (1/39) of children, the patient was therefore excluded from the program. One patient (2.6%, 1/39) from the children group underwent surgery of the TL while included in program – clear assessment of the efficacy of selumetinib treatment itself was not possible. After two courses, response to treatment qualified as CPR in 26.3% (5/19) of adolescents and 20.5% (8/39) of children (p =non-significant).

Third evaluation was conducted in 11 patients from the adolescents’ group and 25 from the children group. In adolescents and children 18.2% (2/11) and 56.0% (14/25) of patients experienced PR, respectively. SD was observed in 72.7% (8/11) of adolescents and 44.0% (11/25) of children. In both groups no patient

Table II. Response to treatment in adolescents and children.

Time point	Response	Adolescents	Children	p -Value
Assessment after 1 st course of therapy	Overall	n=24	n=62	
	SD	62.5% (n=15)	69.4% (n=43)	0.8
	PR	33.3% (n=8)	30.6% (n=19)	
	PD	4.2% (n=1)	0.0% (n=0)	
Assessment after 2 nd course of therapy	Overall	n=19	n=39	
	SD	57.9% (n=11)	53.8% (n=21)	0.5
	PR	31.6% (n=6)	41.0% (n=16)	
	PD	10.5% (n=2)	2.6% (n=1)	
	Underwent TL surgery	0.0% (n=0)	2.6% (n=1)	
	Assessment after 3 rd course of therapy	Overall	n=11	n=25
SD	72.7% (n=8)	44.0% (n=11)		
PR	18.2% (n=2)	56.0% (n=14)		
PD	0.0% (n=0)	0.0% (n=0)		
PR in old TL	9.1% (n=1)	0.0% (n=0)		
SD in new TL				

SD: Stable disease; PR: partial response; PD: progression disease; TL: target lesion.

experienced PD. In one adolescent (9.1%, 1/11) a new TL was observed (the volumetric assessment indicated PR in the old TL and SD in the new one). Response at the third time point was significantly better in children vs. adolescents (OR=5.7; 95% CI=1.02-32; p =0.035, c^2 -test; p =0.067, Fischer exact test). Response to treatment qualified as CPR was observed in 18.2% (2/11) of adolescents and 52.0% (13/25) of children (p =0.057).

Adverse effects. In adolescents the most common adverse effect of selumetinib treatment was acne-like rash, presented by 71.9% (23/32) (Table III). Paronychia, hair loss and kinase creatinine elevation occurred each in 31.3% patients (10/32), whereas hair color change occurred in 28.1% (9/32). Temporary selumetinib dose reduction was necessary in five patients (15.6%), whereas permanent dose reduction was required in one case (3.1%).

In children the most common adverse effect was kinase creatinine elevation present at 43.0% of patients

Table III. Adverse effects in adolescents and children.

Adverse effect*	Adolescents (n=32)	Children (n=79)
Acne-like rash	71.9% (n=23)	34.2% (n=27)
Grade 1	43.8% (n=14)	26.6% (n=21)
Grade 2	25.0% (n=8)	6.3% (n=5)
Grade 3	3.1% (n=1)	1.3% (n=1)
Paronychia	31.3% (n=10)	27.8% (n=22)
Grade 1	18.8% (n=6)	21.5% (n=17)
Grade 2	6.3% (n=2)	5.1% (n=4)
Grade 3	6.3% (n=2)	1.3% (n=1)
Hair loss	31.3% (n=10)	20.3% (n=16)
Grade 1	25.0% (n=8)	20.3% (n=16)
Grade 2	6.3% (n=2)	0.0% (n=0)
Grade 3	0.0% (n=0)	0.0% (n=0)
Kinase creatinine elevation	31.3% (n=10)	43.0% (n=34)
Grade 1	25.0% (n=8)	36.7% (n=29)
Grade 2	6.3% (n=2)	5.1% (n=4)
Grade 3	0.0% (n=0)	1.3% (n=1)
Hair color change	28.1% (n=9)	20.3% (n=16)
Grade 1	28.1% (n=9)	20.3% (n=16)
Grade 2	0.0% (n=0)	0.0% (n=0)
Grade 3	0.0% (n=0)	0.0% (n=0)

*Additional symptoms occurred in single patients from the adolescent group (hypertrichosis, peripheral edema, hematuria, rectal bleeding, nausea and vomiting, cardiotoxicity) and the children group (headaches, diarrhea, and hepatic toxicity).

(34/79). Acne-like rash occurred in 34.2% (27/79), paronychia in 27.8% (22/79), and hair color change and hair loss in 20.3% each (16/79). Three patients (3.8%) required temporary reduction of selumetinib dose, none of them permanently.

Discussion

In this study we have shown that the response to selumetinib therapy was achieved faster in younger patients than in adolescents with NF1-related plexiform neurofibromas. This has not been previously analyzed. The SPRINT study, based on which selumetinib was approved by the Food and Drug Administration and EMA for the treatment of unresectable NF1-related plexiform neurofibromas in pediatric patients, began in 2011. The first results published in 2016-2022 confirmed the drug's efficacy, resulting in PR of tumors in >70% of the study participants (12-14). The maximum effect of the drug was

observed after an average of 18-24 months of therapy, followed by tumor stabilization. Our study includes patients treated for a maximum of 19 months, of which only 36 individuals received the drug for three courses (12-18 months). The Polish results show that over time there is an increase in the number of patients with PR, which is consistent with the observations from the SPRINT study.

In 2023, Gross *et al.* published a study assessing the long-term safety and efficacy of selumetinib (6). The study demonstrated that the following factors did not influence selumetinib treatment outcomes: (i) patient age and sex, (ii) tumor location and size, (iii) tumor progression or lack of progression at the time of treatment initiation, and (iv) dose reduction due to adverse events. In our study, we only assessed the correlation between age and treatment response and observed a better response after three courses of therapy in children vs. adolescents (56.0% vs. 18.2%). Further follow-up of our study group will determine whether the response to the drug is age-dependent or whether age influences the time to achieve PR in patients.

MEK inhibitor therapy is associated with numerous complications, primarily skin-related. To meet the needs of our patients, Polish NF1 experts have published recommendations for the treatment of skin complications following selumetinib, which have been shown to be highly effective and rapidly eliminate side effects (15). It appears that prompt initiation of supportive therapy to prevent skin complications eliminates the need for dose reduction, which had to be implemented in isolated cases in our study.

Gross *et al.* demonstrated that selumetinib treatment often requires many years to maintain tumor response. In those who remain on treatment, both the tumor response to selumetinib and the improvement in tumor-related pain are maintained. No new safety signals were identified; however, known adverse events associated with selumetinib can develop years after initiation of treatment, therefore ongoing monitoring is necessary while patients continue to receive the drug.

Both previously published results regarding selumetinib therapy and our observational study demonstrate that

selumetinib treatment in children with symptomatic, unresectable PN associated with NF1 is safe, tolerable, and often provides durable clinical benefits.

Conclusion

In conclusion, the presented results align with those from the SPRINT study in terms of increasing rate of PR over time. The response to selumetinib therapy is achieved faster in younger children than in adolescents with NF1-related plexiform neurofibromas.

Conflicts of Interest

JnS, AM, AJG, MK, KG, EB, WB, AMM, JW – lecture fees and conference participation from AstraZeneca; KB, JWT – conference participation from AstraZeneca.

Authors' Contributions

JnS, designed the study; JgS, AM, JnS, data analysis; JgS, wrote the manuscript; All Authors – data collection, critical review, manuscript approval.

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Artificial Intelligence (AI) Disclosure

No artificial intelligence (AI) tools, including large language models or machine learning software, were used in the preparation, analysis, or presentation of this manuscript.

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