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Management of NF1-related PNs: Approaches for delivering state-of-the-art care

Practice aid for NF1-PN

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This practice aid is part of an independent medical education activity supported by Alexion and SpringWorks Therapeutics Inc.
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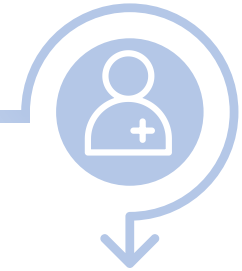
The activity is for healthcare professionals outside of the UK.

Treatment decision algorithm for adult patients with NF1-PNs[1]

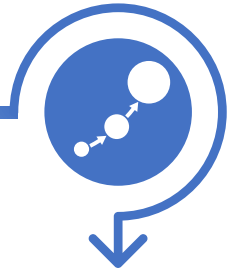
No symptoms
(not located near a vital organ)

- Observe
- Consider annual imaging and clinical evaluation



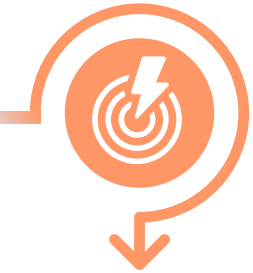
No symptoms
(located near a vital organ)

Low risk of growth
- Consider observation with annual imaging and clinical evaluation



Progression

- Consider further imaging
- Biopsy to evaluate for malignant transformation



Pain

Chronic
- Consider treatment*

New
- Consider further imaging
- Biopsy to evaluate for malignant transformation

Deformity

- Consider treatment*
- Possible consultation with a plastic surgeon

Organ compromise

- Initiate treatment with preference for surgical intervention first*
- Consider concurrent evaluation for malignant transformation

***Decisions should be individualized, weighing risks and benefits in a multidisciplinary setting. For patients in whom surgery is not an appropriate option, medical management is preferred. Urgent specialist MDT/surgical assessment is required for organ compromise, rapid clinical deterioration, or suspected malignant transformation.**

Approved MEK inhibitors to manage symptomatic NF1-PNs in adults

Selumetinib (approved in 2020)[2,3]	
Indication	Adult and pediatric patients ≥1 year (FDA) or 3–18-year-old patients (EMA) with NF1 who have symptomatic, inoperable PNs.
Dosing (based on BSA)	**Capsules**: swallowed whole, 25 mg/m^2 orally BID, with or without food (see PI/SmPC for details), until disease progression. **Granules** (USA only): sprinkled onto or mixed with soft food, 25 mg/m^2, orally BID (see PI for details). **Moderate hepatic impairment (Child–Pugh B):** reduce dosing to 20 mg/m^2, orally BID; recommended dosing not established for severe hepatic impairment (Child–Pugh C). **Coadministered with strong or moderate CYP3A4 inhibitors or fluconazole:** reduce dosing as per PI/SmPC.
Agent-specific warning	**Risk of bleeding** (capsules only; contain vitamin E): risk of bleeding if recommended or safe limits are exceeded, or if patients are co-administered vitamin-K antagonists or anti-platelet agents.

Mirdametinib (approved in 2025)[4,5]	
Indication	Adult and pediatric patients ≥2 years with NF1 who have symptomatic PNs not amenable to complete resection.
Dosing (based on BSA)	2 mg/m^2 (maximum 4 mg) swallowed whole, taken orally BID with or without food for the first 21 days of each 28-day cycle. Continue until progression. Available as capsules or tablets for oral suspension.

Key monitoring and safety considerations for selumetinib and mirdametinib[2–5]	
Cardiac	Assess LVEF before treatment and monitor during treatment in accordance with the relevant PI/SmPC.
Ocular	Arrange a baseline ophthalmologic assessment; repeat assessment during treatment in accordance with the relevant PI/SmPC. Arrange urgent ophthalmologic review for new visual symptoms.
Skin	Monitor for dermatologic adverse reactions, including rash. Provide prompt supportive care and manage dose interruption or modification in accordance with the relevant PI/SmPC and clinical severity.
GI toxicity	Diarrhea, nausea, and vomiting may occur. Assess and manage promptly in accordance with the relevant PI/SmPC.
Increased CPK	Monitor and manage in accordance with the relevant PI/SmPC; promptly assess muscle symptoms or suspected rhabdomyolysis.
Embryo–fetal	Discuss potential fetal risk with patients of reproductive potential and advise effective contraception in accordance with the relevant PI/SmPC.

Management of adult patients with NF1-PNs within an MDT[6,7]

NF1 is a cancer predisposition syndrome that requires systematic medical follow-up, ideally in a cancer center with access to a range of specialists so that patients can readily transition from routine surveillance to active cancer care as needed

NF1 specialist team*

- Coordinates MDT and community-based care for adults with NF1-PNs
- Identifies a named clinical lead and appropriate point of contact
- Facilitates communication, follow-up, and referral between the patient, MDT and community care

Specialist team

Review all patient information

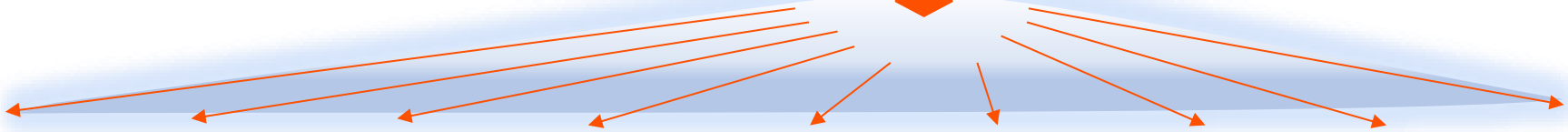
NF1 specialist

Expert

- Understands the complexities of NF care and ensures patient care needs are effectively coordinated
- Shares nuanced knowledge with subspecialist referrals

Pre-clinic huddle
Set preliminary care plans

Post-clinic debrief
Consolidate next steps



Genetics | **Neurosurgery or neurology** | **Medical oncology** | **Social work** | **Plastic surgery** | **Pain management** | **Surgical oncology** | **Primary care** | **Patient advocacy organization**

Robust pain management is critical

- In addition to physical suffering, pain causes psychological distress and leads to psychosocial dysfunction

Social work support

- Addresses the psychosocial, psychiatric, financial, self-image-related, and work-related stressors

Plastic surgery

- Used to treat painful or disfiguring cutaneous neurofibromas

The MDT

- Complements, rather than replaces, existing community-based care

Community-care providers

- Structured pediatric-to-adult transition to the community-care system is critical
- Comprehensive MDT summary notes following consultations with adults facilitate clear communication with providers
- Intentional community outreach enhances the relationship between the NF1 MDT and the community-care providers

*Care coordination may be delivered by a nurse coordinator, designated clinician, or other member of the specialist NF1 team, according to local resources.

MDT checklist for each review[1,6,8]

To do...

- **Review** tumor status and the patient's pain, function, and HRQoL
- **Identify** clinical or radiologic features that may suggest malignant transformation or organ compromise
- **Agree** an individualized management and monitoring plan, including the responsible clinical lead
- **Communicate** the agreed plan clearly with the patient and relevant community-care providers

Abbreviations and references

Abbreviations

BID, twice a day; BSA, body surface area; CPK, creatine phosphokinase; CYP3A4, cytochrome P450 3A4; EMA, European Medicines Agency; FDA, US Food and Drug Administration; GI, gastrointestinal; HRQoL, health-related quality of life; LVEF, left ventricular ejection fraction; MDT, multidisciplinary team; MEK, mitogen-activated protein kinase; NF1, neurofibromatosis type 1; PI, prescribing information; PN, plexiform neurofibroma; SmPC, summary of product characteristics.

References

1. Armstrong AE, et al. *Neuro Oncol*. 2026;28:847–63.
2. FDA. Selumetinib PI. Available at: https://bit.ly/4bYjYGh.*
3. EMA. Selumetinib SmPC. https://bit.ly/46kR53U.*
4. FDA. Mirdametinib PI. Available at: https://bit.ly/4wzXB1r.*
5. EMA. Mirdametinib SmPC. Available at: https://bit.ly/3U7H1IE.*
6. Lipinski LJ, et al. *Orphanet J Rare Dis.* 2026;21:219.
7. Lalvani S, Brown RM. *J Multidiscip Healthc*. 2024;17:1803–17.
8. Carton C, et al. *EClinicalMedicine*. 2023;56:101818.

***All SmPCs and PIs were accessed on September 5, 2026.**

The guidance provided by this practice aid is not intended to directly influence patient care. Clinicians should always evaluate their patients' conditions and potential contraindications and review any relevant manufacturer product information or recommendations of other authorities prior to consideration of procedures, medications, or other courses of diagnosis or therapy included here.

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