A Phase 2, Open-Label Study to Evaluate the Safety and Effects of HLX-1502 in Patients With Neurofibromatosis Type 1

NCT06541847

Status	RECRUITING
Phase	Phase 2
Sponsor	Healx Limited
Enrollment	25 participants

Key Eligibility Criteria

Inclusion (11)

- All participants must have a diagnosis of NF1 based on the 2021 revised consensus criteria.
- Participants must have PN(s) that are progressive OR are causing significant morbidity, such as (but not limited to) head and neck lesions that are compromising the airway or great vessels, brachial or lumbar plexus lesions that are causing nerve compression and loss of function, lesions causing significant disfigurement (e.g., orbital lesions), lesions of the extremity that cause limb hypertrophy or loss of function, and painful lesions. Participants with paraspinal PN will be eligible for this trial. Histologic confirmation of tumor is not necessary but should be considered if there are clinical or radiographic findings concerning for malignant transformation of a PN.
- Measurable Disease: Participants must have measurable PN(s) amenable to volumetric MRI analysis. For the purpose of this study, the target lesion must be seen on at least 3 consecutive MRI slices and the field of view must contain the entire tumor of interest. Tumors must be at least 3 mL in volume (most PN 3 cm in longest diameter will meet this criteria). If the tumor is \< 3 cm in longest diameter, the participant may still be eligible. Central review of the MRI of the target PN is required prior to enrollment to ensure that the tumor is measurable and amenable to volumetric analysis.
- Age: Participants must be e 12 years of age at the time of study entry. Note: Although prior MEKi therapy is not a requirement, patients should be counseled on the availability of FDA-approved MEKi therapies prior to enrollment.
- Weight e 42 kg.

... and 6 more (see full listing online)

Exclusion (18)

- Prior treatment with HLX-1502 for a PN.
- The participant has used any of the following systemic medications/ therapies within the specified period prior to enrollment: MEK-inhibitors, other drugs in the TKI class, HLX-1502, Participants may have received treatment for a PN or other tumor/malignancy but must have fully recovered to baseline or CTCAE d Grade 1 from acute toxicities from prior therapies except alopecia, Myelosuppressive chemotherapy, Hematopoietic growth factors, Biologic (anti-neoplastic agent), Investigational Drugs, Any other systemically administered anti-neoplastic agent and Radiation therapy.
- Evidence of an NF1-related tumor such as optic pathway or other low-grade glioma, high-grade glioma, malignant peripheral nerve sheath tumor, or other cancer/tumor requiring treatment with chemotherapy, biologic therapy, surgery or radiation therapy.
- Participants with high-grade glioma, malignant peripheral nerve sheath tumor, or other malignancy who received treatment in the last 12 months. Exceptions include basal cell carcinoma of the skin and squamous cell carcinoma of the skin that have undergone potentially curative therapy. If the investigator has any clinical concerns for ANNUBP/Atypical Neurofibroma or MPNST, a biopsy sample must be taken prior to study confirming eligibility.
- Dental braces or prosthesis that interfere with volumetric analysis of the neurofibroma(s).

... and 13 more (see full listing online)

Locations (13 total)

University of Alabama at Birmingham, Birmingham, Alabama, United States
Children's Hospital Los Angeles, Los Angeles, California, United States
Children's Hospital Colorado, Aurora, Colorado, United States
... and 10 more locations

https://clinicaltrials.gov/study/NCT06541847