Phase 1 study of selumetinib in Chinese pediatric and adult patients (pts) with neurofibromatosis type 1 (NF1) and inoperable pleomorphic neurofibromas (PN): Interim results

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OBJECTIVE

• Selumetinib may address an unmet medical need for patients with NF1 and inoperable PN in China

• Based on these interim analysis results from an ongoing Phase 1 study, selumetinib at a dosage of 25 mg/m² twice daily was well tolerated with a manageable safety profile in both pediatric and adult patients with NF1 and inoperable PN in China

In China, patients with NF1 and inoperable PN have a considerable unmet need for medical treatments. Hence, we performed a Phase 1, dose-escalation study to evaluate the safety and tolerability of selumetinib in Chinese patients with NF1 and inoperable PN. Selumetinib, an orally available selective MEK1/2 inhibitor, is the only MEK1/2 inhibitor approved by the U.S. Food and Drug Administration (FDA) for use in patients with NF1. The study was designed to identify recommended dosages for further development of selumetinib in pediatric and adult patients with NF1-PN. This report presents the interim results from the ongoing study, which includes 4 pediatric and 12 adult patients enrolled in the study, with a median follow-up of 6.7 months.

BACKGROUND

Neurofibromatosis type 1 (NF1) is a genetic condition where some people living with it develop neurofibromas, which are benign tumors that can grow in any nerve. Symptoms of NF1 can include café-au-lait macules, freckling in axilla or groin, ≥6 café-au-lait macules, or ≥2 neurofibromas in the axilla or inguinal regions. Patients with NF1-PN have a considerable unmet need for medical treatments. Therefore, we performed a Phase 1, dose-escalation study to evaluate the safety and tolerability of selumetinib in Chinese patients with NF1 and inoperable PN. This study is the first of its kind to assess the efficacy of selumetinib in Chinese NF1-PN patients.

STUDY DESIGN AND METHODS

Selumetinib is an orally available selective MEK1/2 inhibitor approved by the U.S. Food and Drug Administration for use in patients with NF1. The study was designed to identify recommended dosages for further development of selumetinib in pediatric and adult patients with NF1-PN. This report presents the interim results from the ongoing study, which includes 4 pediatric and 12 adult patients enrolled in the study, with a median follow-up of 6.7 months.

PLAIN LANGUAGE SUMMARY

Why did we perform this research?

Neurofibromatosis type 1 (NF1) is a genetic condition where some people living with it develop neurofibromas, which are benign tumors that can grow in any nerve. Symptoms of NF1 can include café-au-lait macules, freckling in axilla or groin, ≥6 café-au-lait macules, or ≥2 neurofibromas in the axilla or inguinal regions. Patients with NF1-PN have a considerable unmet need for medical treatments. Therefore, we performed a Phase 1, dose-escalation study to evaluate the safety and tolerability of selumetinib in Chinese patients with NF1 and inoperable PN. This study is the first of its kind to assess the efficacy of selumetinib in Chinese NF1-PN patients.

How did we perform this research?

This is a Phase 1, dose-escalation study conducted in China to assess the safety and tolerability of selumetinib in Chinese patients with NF1 and inoperable PN. The study was designed to identify recommended dosages for further development of selumetinib in pediatric and adult patients with NF1-PN. This report presents the interim results from the ongoing study, which includes 4 pediatric and 12 adult patients enrolled in the study, with a median follow-up of 6.7 months.

What were the findings of this research and what are the implications?

In both children and adults, selumetinib was able to shrink the size of their NF1-PN, and side effects were generally manageable. The study showed that selumetinib could be used to treat NF1-PN in children and adults, with a median follow-up of 6.7 months. The study concluded that selumetinib is well tolerated and has a manageable safety profile in both pediatric and adult patients with NF1 and inoperable PN, indicating that it could be a promising treatment option for these patients.