patient resource

Tirabrutinib for the treatment of relapsed or refractory primary central nervous system lymphoma: Efficacy and safety from the phase II PROSPECT study

WHAT IS PCNSL?

Primary central nervous system lymphoma (PCNSL) is a rare and aggressive type of non-Hodgkin lymphoma that starts in the brain, spinal fluid, or eyes. Treatment options are limited, especially for patients whose disease has returned (relapsed) or hasn't responded to previous treatment (refractory).

As a patient advocacy organization, the End Brain Cancer Initiative is proud to support awareness and education around the PROSPECT Study. Our goal is to connect patients with emerging treatment options and ensure they have access to clear, actionable information about clinical trials like this one.

WHAT IS TIRABRUTINIB?

Tirabrutinib is an oral (taken by mouth) targeted therapy. It works by blocking a protein called BTK (Bruton's tyrosine kinase), which plays a role in cancer cell growth. While it's already approved for PCNSL in Japan, Taiwan, and South Korea, it is still being studied in the U.S.

ABOUT THE PROSPECT STUDY

- **Purpose:** To test how safe and effective tirabrutinib is in U.S. patients with relapsed (came back after treatment) or refractory (didn't respond to treatment) PCNSL.
- **How it was given:** One 480 mg pill daily until the cancer worsened or side effects became too severe.
- **Number of patients:** 48
- **Average age:** 65 years old (ranging from 34 to 87)

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WHAT DID THE STUDY FIND? (PHASE II RESULTS)

Effectiveness

- 66.7% of patients (about 2 out of 3) responded to the treatment.
  - 43.8% had complete response (cancer gone or almost gone).
  - 22.9% had partial response (tumor shrank but didn't disappear).
- Patients responded quickly – on average within 1 month.
- For those who responded, the benefit lasted around 9 months.

Side Effects

Most patients (about 98%) experienced some side effects.
Common mild to moderate side effects included:

- Tiredness
- Rash
- Itching
- Low red or white blood cells (like anemia or low neutrophil counts)

Serious side effects (grade 3 or higher) happened in about half of the patients, most commonly:

- Low white blood cells
- Severe skin rash

Two patients died during the study, but these deaths were not directly related to the drug.

Conclusion

Tirabrutinib may offer a new treatment option for patients with relapsed or refractory PCNSL. It showed promising results in shrinking tumors and had a manageable safety profile. More studies are ongoing to confirm its benefit and bring it closer to approval in the U.S.

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Visit EndBrainCancer.org/Ono-Pharma or call 425-436-8688 to speak with our team about this study or other treatment options.

CITATION

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This factsheet is intended to provide a comprehensive overview of the efficacy and safety from the phase II PROSPECT study of tirabrutinib for the treatment of relapsed or refractory primary central nervous system lymphoma. For personalized medical advice, always consult with your healthcare provider.

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