

Community Update:

Alpelisib in Pediatric and Adult Patients with PIK3CA-Related Overgrowth Spectrum (PROS)

EPIK-P4 | [ClinicalTrials.gov ID: NCT06997588](https://clinicaltrials.gov/ct2/show/study/NCT06997588)

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Dear PROS Community,

We would like to share that the EPIK-P4 study is currently recruiting in multiple countries, including the United States, Australia, Austria, Belgium, France, Germany, Italy, Netherlands Spain, Switzerland, and the United Kingdom. In the United States, sites are open across Missouri, North Carolina, Ohio, Pennsylvania, and Texas.

EPIK-P4 is a Novartis-sponsored clinical research study for children, adolescents, and adults living with PIK3CA-Related Overgrowth Spectrum, also known as PROS. The study is designed to demonstrate efficacy and assess the safety, tolerability, and pharmacokinetics of oral daily alpelisib, an inhibitor of PI3KCA which has an activated mutation in PROS lesions.

Tolerability refers to how well participants can take the medicine, including whether side effects are manageable.

Pharmacokinetics, or PK, refers to how the medicine moves through the body and how the body processes it.

What is EPIK-P4?

EPIK-P4 is a Phase II, single-arm, open-label clinical research study sponsored by Novartis. Single-arm means that all participants in the study receive the study medicine, alpelisib, according to the study protocol. Open-label means that participants and the study team know which medicine is being given.

What is the purpose of the study?

The purpose of EPIK-P4 is to help researchers better understand alpelisib in people living with PROS. The study will look at:

- Changes in PROS-related lesions.
- Safety and tolerability of treatment.
- How the medicine is processed by the body.

How will the study work?

The study includes a screening period, a 48-week core treatment period, two extension periods, and post-trial access for participants who may continue to benefit from treatment.

- Screening period: up to 42 days. During this period, potential participants are assessed to see whether they may be eligible. Screening includes a whole-body MRI scan to evaluate PROS-related lesions.
- Core treatment period: 48 weeks. Participants who meet the study requirements receive alpelisib once daily by mouth. Adults start at 250 mg once daily, with no dose escalation allowed. Children and adolescents start at age-based doses according to the study protocol: 50 mg once daily for participants aged 2 to under 6 years, and 125 mg once daily for participants aged 6 to under 18 years.
- Extension 1 period: participants in both groups continue treatment under the same rules as the core period. This period lasts until Week 168 after each participant completes the core period.

- Extension 2 period: participants who complete Extension 1 before the end of the study may continue treatment under the same rules as the core and Extension 1 period until the last participant completes Extension 2.

Participants who are still deriving clinical benefits from alpelisib at the end of the study may receive post-trial access to alpelisib.

What are the key criteria for participation?

- Male or female participants aged 2 years or older at the time of informed consent or assent.
- A diagnosis of PROS with symptomatic and progressive overgrowth.
- Documented evidence of a somatic PIK3CA gene mutation, based on a DNA test performed in a local laboratory.
- Adequate overall health status and organ function, as assessed by the study team.
- At least one measurable PROS-related lesion that is associated with symptoms or functional limitations affecting everyday life.

Key exclusion criteria

- People whose only PROS-related features are isolated macrodactyly, epidermal nevus or nevi, and/or macroencephaly, without other PROS-related lesions.
- Previous treatment with alpelisib or another PI3K inhibitor, except for a short treatment attempt that meets the protocol-defined conditions.
- Major surgery within 3 months before informed consent or assent.
- Radiation exposure for PROS treatment purposes within 12 months before informed consent or assent.
- Recent clinically meaningful bleeding from a PROS-related lesion.
- Worsening PROS-related symptoms or test results during screening suggesting the condition is not well controlled.
- Certain recent PROS-related complications, such as clinically meaningful thrombotic events, bleeding, or procedures for vascular complications, as defined in the study criteria.

What may participation involve?

- Study visits at a participating clinical research center.
- Screening and eligibility assessments.
- Study treatment with oral daily alpelisib, if eligible.
- Imaging or other assessments of PROS-related lesions.
- Safety monitoring by the study team during participation.

The participating study site can provide more details about the exact assessments and visit schedule.

Where can I find additional information?

Study details, eligibility criteria, and participating locations can be found on the ClinicalTrials.gov study page: Study Details for [NCT06997588](https://clinicaltrials.gov/study/NCT06997588). Patients and families interested in learning more should speak with their healthcare provider or contact a participating study site.

Kind regards,

The Novartis EPIK-P4 Study Team

This statement is not intended to establish any legally enforceable rights, obligations, or commitments on Novartis

References

ClinicalTrials.gov. NCT06997588. Available at: <https://clinicaltrials.gov/study/NCT06997588>.

<https://clinicaltrials.gov/study/NCT06997588?cond=PROS&viewType=Card&rank=1>. Accessed August 2026.

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