ForPatients
by Roche

CNS TumorsSolid TumorsALK Fusion-positive Solid Tumors

A clinical trial to look at how safe alectinib is, how the body processes it and how well it works in children and young people that have a solid tumour in the brain, body or spine that has a change in the ALK gene and did not respond to previous treatment or has no treatment options available

A Study Evaluating the Safety, Pharmacokinetics, and Efficacy of Alectinib in Pediatric Participants With ALK Fusion-Positive Solid or CNS Tumors

Trial Status	Trial Runs In	Trial Identifier
Recruiting	12 Countries	NCT04774718 2023-504084-17-00 2020-004239-25 GO42286

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase I/II open-label, multicenter study evaluating the safety, pharmacokinetics, and efficacy of alectinib in pediatric patients with ALK fusion-positive solid or CNS tumors for whom prior treatment has proven to be ineffective or for whom there is no satisfactory treatment available

Trial Summary:

This study will evaluate the safety, pharmacokinetics, and efficacy of alectinib in children and adolescents with ALK fusion-positive solid or CNS tumors for whom prior treatment has proven to be ineffective or for whom there is no satisfactory standard treatment available.

Hoffmann-La Roche	Phase 1/Phase 2
Sponsor	Phase

NCT04774718 2023-504084-17-00 2020-004239-25 GO42286
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 17 Years	No

1. Why is the iMATRIX-alectinib clinical trial needed?

Solid tumours are cancers that grow in any of the body's organs or tissues. Many treatments are available for children and young people with cancer. But sometimes these treatments do not work or the cancer comes back after treatment. New drugs, such as alectinib, might help people with cancer that has come back or did not respond to treatment, or who have no other treatments available to them. Alectinib may stop cancer from growing and spreading to other parts of the body. It works by targeting cancer cells that have certain changes in the *ALK* gene (called *ALK* gene fusion). Alectinib is an experimental drug, which means health authorities have not approved alectinib for treating children or young people with cancer.

This clinical trial aims to test how safe alectinib is at different doses and how well it works in children and young people. The trial will also look at how the body breaks down and gets rid of alectinib.

2. How does the iMATRIX-alectinib clinical trial work?

This clinical trial is recruiting children and young people with an *ALK* gene fusion solid tumour. People can take part if their cancer has come back or did not respond to treatment, or they have no other treatment options available to them. People who take part in this clinical trial (participants) will be given the clinical trial treatment alectinib for as long as it can help them. Or until they have unacceptable side effects or the trial is stopped. The clinical trial doctor will see them every week for the first month, then every 2 weeks for 5 months and then every month while being given treatment. Some visits may be with a mobile nurse in the participants' home if the participant agrees to it. The visits with the clinical trial doctor or mobile nurse will include checks to see how the participant responds to the treatment and any side effects they may have. After the last dose of treatment, participants will be seen for a follow-up appointment 1 month later. Then every 3 months at clinic visits, by telephone or through their medical records, for as long as they agree to it. Total time of participation in the clinical trial will be about 5 years, depending on when they join the trial. Participants can stop trial treatment and leave the clinical trial at any time.

3. What are the main endpoints of the iMATRIX-alectinib clinical trial?

The main clinical trial endpoints (the main results measured in the trial to see how safe the drug is, how the body processes it and how well the drug works) are:

- The number of intolerable side effects that occur during the first month of treatment
- The number and seriousness of side effects
- The number of participants whose tumours shrink or disappear on scans
- How the body breaks down and gets rid of alectinib

The other clinical trial endpoints include:

- How long a participant responds to treatment before their cancer gets worse
- The amount of time between starting treatment and responding to treatment
- The number of participants whose tumours disappear, get smaller, or stay the same after
  6 months of treatment
- The amount of time between the start of trial treatment and participants' cancer getting worse
- How long participants live

4. Who can take part in this clinical trial?

People can take part in this trial if they are less than 18 years old and have an *ALK* gene fusion solid tumour in the body, brain or spine. People can also take part if their cancer has come back or did not respond to treatment. Or they have no other treatment options available for them.

People may not be able to take part in this trial if they:

- Have already received *ALK* treatment
- Have certain lymphomas
- Are bed bound or require special care and assistance
- Have received certain other treatments such as a stem-cell infusion or an organ transplant
- Have/had certain medical conditions such as infections, liver disease, bone disorders, or gut disorders that may stop alectinib being absorbed by the body properly
- Are receiving another treatment for their cancer and not just the treatment provided in this trial
- Are pregnant or breastfeeding, or are planning to become pregnant within 3 months after the last dose of alectinib

5. What treatment will participants be given in this clinical trial?

Everyone who joins this clinical trial will be given alectinib as capsules to be swallowed twice a day with food for as long as it can help them. Or until they have unacceptable side effects or the trial is stopped. Participants who struggle to swallow full capsules can be given alectinib as a liquid to drink (a suspension prepared with the capsule) or through a feeding tube.

This is an open-label trial, which means everyone involved, including the participant and the clinical trial doctor, will know the clinical trial treatment the participant has been given.

6. Are there any risks or benefits in taking part in this clinical trial?

The safety or effectiveness of the experimental treatment or use may not be fully known at the time of the trial. Most trials involve some risks to the participant. However, it may

not be greater than the risks related to routine medical care or the natural progression of the health condition. People who would like to participate will be told about any risks and benefits of taking part in the clinical trial, as well as any additional procedures, tests, or assessments they will be asked to undergo. All of these will be described in an informed consent document (a document that provides people with the information they need to decide to volunteer for the clinical trial).

Risks associated with the clinical trial drug

Participants may have side effects (an unwanted effect of a drug or medical treatment) from the drug used in this clinical trial. Side effects can be mild to severe, even life-threatening, and vary from person to person. Participants will be closely monitored during the clinical trial; safety assessments will be performed regularly.

Participants will be told about the known side effects of alectinib and possible side effects based on human and laboratory studies or knowledge of similar drugs. Participants will be told about any known side effects of swallowing capsules, taking liquid suspensions or using feeding tubes.

Potential benefits associated with the clinical trial

Participants' health may or may not improve from participation in the clinical trial. Still, the information collected may help other people with similar medical conditions in the future.

For more information about this clinical trial see the **For Expert** tab on the specific ForPatient page or follow this link to ClinicalTrials.gov

Trial-identifier: NCT04774718

Inclusion Criteria:

- Histologically confirmed diagnosis of CNS or solid tumors with documented evidence of ALK gene fusions as assessed centrally through the use of the investigational F1CDx assay or based on pre-existing NGS test results
- Disease status: prior treatment proven to be ineffective (i.e. relapsed or refractory), or for whom there is no satisfactory standard treatment available. Disease should be measurable and evaluable as defined by Response Evaluation Criteria in Solid Tumors (RECIST) v 1.1, or Response Assessment in Neuro-oncology criteria (RANO) +/- bone marrow criteria for primary CNS tumors or International Neuroblastoma Response Criteria (INRC)
- Available tumor tissue for submission to the Sponsor from active disease, obtained subsequent to last anti-cancer therapy regimen administered and obtained prior to study enrollment (preferred option), or archival tumor tissue from original diagnosis, or willingness to undergo a core or excisional biopsy sample collection prior to enrollment
- For participants < 16 years old, Lansky Performance Status >/= 50%
- For participants >/= 16 years old, Karnofsky Performance Status >/= 50%
- Adequate bone marrow function as defined by the protocol within at least 28 days prior to initiation of study drug

- Participant and/or caregiver willingness and ability to complete clinical outcome assessments throughout the study using either electronic, paper, or interviewer methods
- For females of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use contraception, and agreement to refrain from donating eggs, as defined by the protocol
- For males who are not surgically sterile: agreement to remain abstinent (refrain from heterosexual intercourse) or use contraception, and agreement to refrain from donating sperm, as defined by the protocol

Exclusion Criteria:

- Medical history of: prior use of ALK inhibitors; diagnosis of Anaplastic Large Cell Lymphoma (ALCL); any gastrointestinal disorder that may affect absorption of oral medications, such as mal-absorption syndrome or status post-major bowel resection; history of organ transplant; stem cell infusions as defined by the protocol
- Substance abuse within 12 months prior to screening
- Familial or personal history of congenital bone disorders, bone metabolism alterations, or osteopenia
- Treatment with investigational therapy 28 days prior to initiation of study drug
- Liver or kidney disease as defined by the protocol
- National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE) v5.0 grade >/=3 toxicities attributed to any prior therapy such as radiotherapy (excluding alopecia), which have not shown improvement and are strictly considered to interfere with alectinib
- Co-administration of anti-cancer therapies other than those administered in this study
- Active hepatitis B or C virus (HBV, HBC), or known HIV-positivity or AIDS-related illness
- Any clinically significant concomitant disease or condition that could interfere with, or for which the treatment might interfere with, the conduct of the study or the absorption of oral medications or that would, in the opinion of the Principal Investigator, pose an unacceptable risk to the participant in this study
- Any psychological, familial, sociological, or geographical condition potentially hampering compliance with the study protocol requirements and/or follow-up procedures; such conditions should be discussed with the participant before trial entry
- Planned procedure or surgery during the study except as permitted treatment as defined by the protocol
- Infection considered by the investigator to be clinically uncontrolled or of unacceptable risk to the participant upon induction of neutropenia, including participants who are, or should be, on antimicrobial agents for the treatment as active infection
- Pregnant or breastfeeding, or intending to become pregnant during the study or within 3 months after the final dose of alectinib