Summary of Results for Laypersons

Astellas is grateful to the children who took part in this clinical study. Thank you.

What was the Study Called?

Open-label Phase 2 Study of Single-agent Erlotinib for Patients with Pediatric Ependymoma Previously Treated with Oral Etoposide in Protocol OSI-774-205

Why was this Study Needed?

An ependymoma is a type of brain tumor that begins in ependymal cells. Those are the cells that line the ventricles and passageways in the brain and the spinal cord. They make the fluid that nourishes the brain (cerebrospinal fluid). Ependymomas in children are treated with the experimental medicine etoposide. Ependymomas in some children may come back after the treatment. Or they may become resistant to etoposide. This means that etoposide can no longer stop the cancer growth or keep the cancer stable. Therefore, there was a need to study new treatments for ependymomas in children that are resistant to etoposide. Erlotinib (also known as OSI-774 and Tarceva®) is an experimental oral medicine (taken by mouth) for such ependymomas.

This study was conducted in children with ependymomas. The children took erlotinib. This study looked at how safe it was for them to take erlotinib. It was also important to find out what unwanted effects the children had from the study medicine.

The study started in May 2011. Thereafter, the sponsor of this study (Astellas) found out that children with ependymomas did not benefit from erlotinib treatment. Astellas stopped this study in September 2012. When the study was stopped, 4 children had taken study medicine. Astellas reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was This and Who Took Part in it?

This was an "open-label" study. This means that the parents of each child and the study doctors knew which study medicine that child took (erlotinib).

This study included children with ependymoma. They had taken etoposide in an earlier study (OSI-774-205). Their cancer had gotten worse while taking etoposide. Or they had to stop taking etoposide because they could not tolerate its unwanted effects.

During the study, the study doctor did a check-up of the children at several study visits. At the first visit, children were checked to see if they could be in the study. Children who could be in the study took erlotinib tablets (85 mg for every square meter of body surface) once a day.

The children could take study medicine until their cancer got worse, they died, they had unwanted effects they could not tolerate or they or their parents asked to stop treatment or the study doctor decided to stop treatment.

This study took place at 3 clinics in 2 countries. Four children were in the study and took at least 1 dose of study medicine.

	Number of Children
Age Group	
Aged between 5 and 14 years	4
Sex	
Boys	3
Girls	1
Clinic Location	
European Union Countries (*at the time of the study*)	1
The UK	1
Outside European Union	3
Canada	3

What Were the Study Results?

This study looked at how safe it was for children with ependymomas to take erlotinib. The study looked at the medical problems (called "adverse events") children had.

A lot of research is needed to know whether a medicine causes a medical problem. So when new medicines are being studied researchers keep track of all medical problems that patients have while they are in the study. These medical problems are called "adverse events" and are recorded whether or not they might be caused by the treatment taken.

The 4 children in this study took erlotinib for a median (middle value in a sorted list of numbers) of 91.0 days. It was safe for them to take erlotinib. Their adverse events were similar to the adverse events seen in adults who took erlotinib.

The table below shows the most common adverse events experienced by children who took at least 1 dose of study medicine in this study.

Adverse Event	Erlotinib (out of 4 children)
Any adverse event	4 (100%)
Fatigue or tiredness	3 (75.0%)
Headache or head pain	3 (75.0%)
Cough	2 (50.0%)
Decreased appetite	2 (50.0%)
Sore throat	2 (50.0%)

What Adverse Reactions did Patients Have?

An "adverse reaction" is any medical problem or "adverse event" that is judged by the study doctor to be possibly caused by a medicine or treatment used in the study.

The table below shows the most common adverse reactions experienced by children who took at least 1 dose of study medicine in this study.

Adverse Reaction	Erlotinib (out of 4 children)
Any adverse reaction	4 (100%)
Fatigue or tiredness	2 (50%)

An adverse reaction is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care.

None of the children in this study experienced a serious adverse reaction.

One child died. The death was not because of erlotinib.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study and reflects the information available as of October 2013. You can find this summary and more information about this study online at http://www.astellasclinicalstudyresults.com.

Please remember that researchers look at the results of many studies to find out how well medicines work and which adverse reactions they might cause. This summary only shows the results of this 1 study. Your child's doctor may help you understand more about the results of this study.

Sponsor contact details:

Astellas Pharma Global Development
1 Astellas Way
Northbrook, IL 60062
USA