DEEP-2

DEferiprone Evaluation in Paediatrics

Lay summary

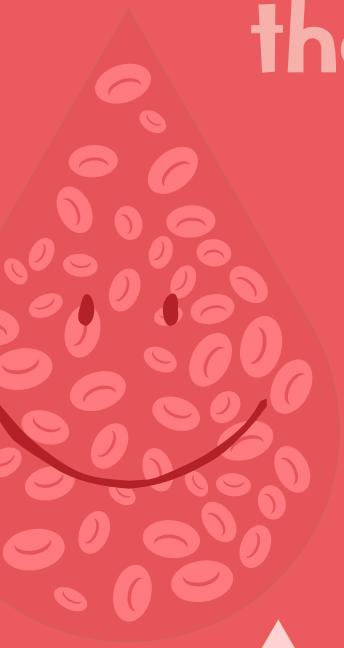
DEEP-2 Study: Mission completed!

Thanks to all the children participating in the **DEEP-2 study**, researchers answered important health questions about the medicine called **deferiprone**. This booklet is for them and for all children who want to better understand the results of this paediatric clinical study.

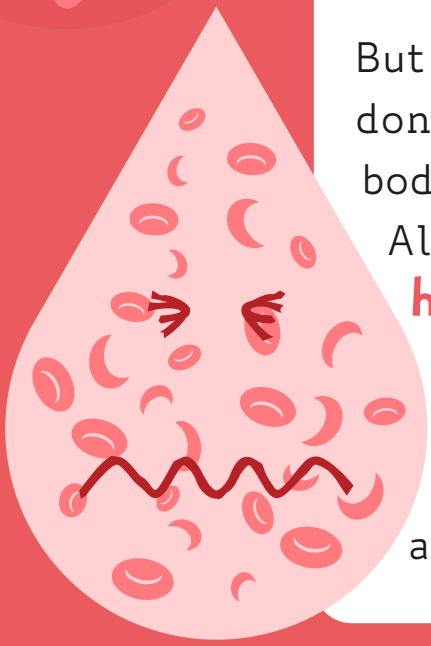
What was DEEP-2 study about?

The DEEP-2 study has been a **trial**, in other words, a way of finding out whether **deferiprone** is helpful and works properly for children with hereditary anaemias, like thalassemia or sickle cell anaemia. Let's see together what the researchers found out about this medicine.

Why was the study needed?



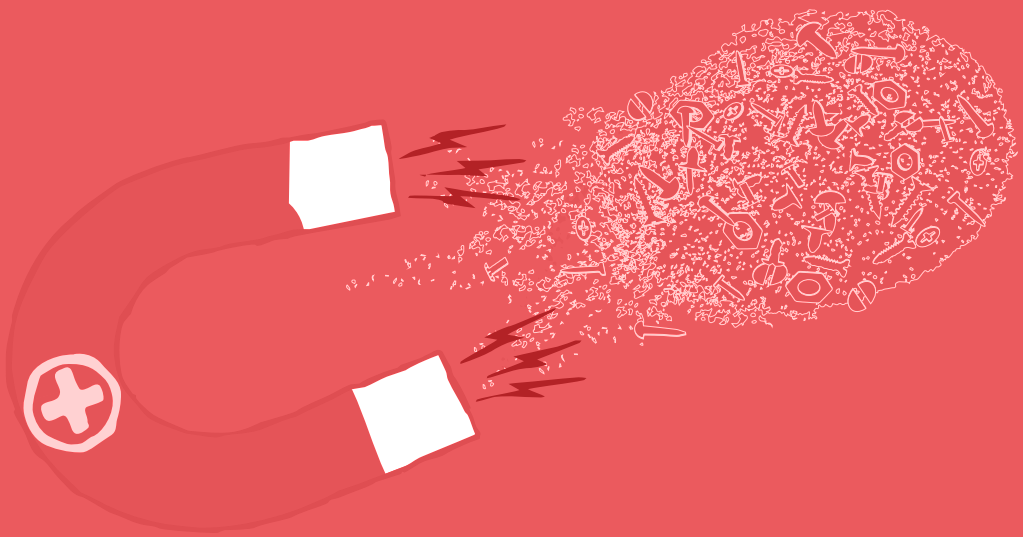
Inside the blood are millions of little soldiers called **red blood cells**. They do an important job because they bring us the **oxygen**, the fuel our body needs to work properly.



But if red blood cells don't work well, our bodies run out of fuel! All because of **hereditary anaemias**, which are a large family of **diseases present at birth** that affect the blood.

That is why you need to have a **blood transfusion**: to help your red blood cells to carry oxygen to every part of your body.

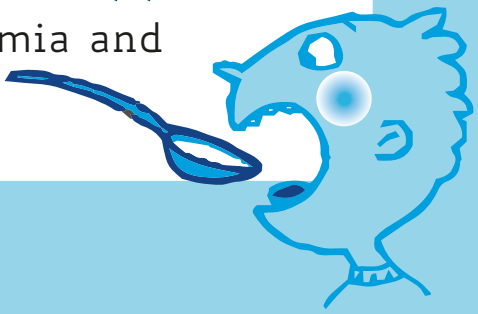
The problem is that every time you have a transfusion, the new blood brings with it **a lot of iron**. If a whole lot of iron particles get together, it spells trouble! **So, we need a medicine that gets rid of iron from the body!**



Which medicine was studied?

Researchers invented different medicines to remove the iron: some needed a needle and a pump, others were pills to swallow or tablets to dissolve in water. Among these was a medicine called **deferiprone**, which was used in people with thalassemia but was not suitable for young children like you!

The DEEP-2 study looked at a new shape of this medicine, a **syrup** with a good taste and easy to use, to find out if it worked properly and was **suitable also for very young children** with thalassemia and other anaemias.



What happened during the study?

393 children and young people under the age of 18 joined the study. They were from Albania, Cyprus, Egypt, Greece, Italy, Tunisia, and the United Kingdom.

These participants were divided into **two groups**: one group received the **syrup deferiprone**, while the other group took **tablets** of the medicine called **deferasirox**, dissolved in water, for **one year**.

The researchers compared deferiprone with another medicine that is already known to be good for kids with hereditary anemias to see if deferiprone works just as well.

How did the doctors know if the medicines works or not?

The children took some special tests. Thanks to these simple tests the doctors checked if the two medicines were actually reducing the **extra iron** in the **blood, heart, liver** of the participants' and overall, their bodies were working well.

These examinations were not painful nor dangerous and were useful to understand if the medicine is safe and works in children.

Sometimes the medicines can cause unwanted effects

What did we learn about Deferiprone?

All the medicines we take for their good effects can sometimes also affect other parts of our body and produce **unwanted effects**. Usually, these effects are not very dangerous, but it is important to investigate them to understand if the medicine is **safe**.

In DEEP-2, researchers studied the unwanted effects that happened when kids took the two medicines, **to see if deferiprone is safe as well as deferasirox in children**.

Some of the participants did not feel well after taking the study medicines. Every time when a participant felt something strange, **the doctors recorded it as unwanted effect**.

During the study, both groups had a similar number of these unwanted effects. For example: some kids had experience with vomiting, nausea, diarrhoea or pain in arms and legs. One of the most important unwanted effects found in the patients was the reduction of **white cells** (these cells are very important for defending our body from infections).

So, then what happened?

Once the study was completed, **researchers compared the effect of the two drugs**. A total 310 patients completed the treatment.

In the end, the study helped to show that the syrup deferiprone, tastier and easier to swallow than the other types of medicines, can be very effective and useful for all young people and children with anaemias who need transfusions.

DEEP-2 study also showed that some bad effects can occur, however they are easy manageable and well known to your doctor. Therefore, **we can say that deferiprone is safe for children of every age**, from very young to 18 years old.

Where can I find more information about this study?

If you are more curious about this study, check this website:

www.deepproject.eu

If you have questions about the study results, you can ask the doctor or the staff at your study site. We are sure that they will be happy to answer them.

DEFERIPRONE EVALUATION IN PAEDIATRICS

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SEVENTH FRAMEWORK PROGRAMME

CVBF

TEDDY NETWORK
European Network of Excellence for Paediatric Research