DEFERIPRONE EVALUATION IN PAEDIATRICS

This project is co-funded by the 7th Framework Programme of the European Union

DEEP-1 STUDY NEWSLETTER
ISSUE 1 - 2013

Dear Investigators and DEEP-1 Study Participants,
WELCOME to the first newsletter of the DEEP-1 study

This edition is devoted to:

1. the announcement of the completion of the first phase of the trial (enrolment of 18 patients)
2. the description of the key steps in the second study phase
3. the update on the study timelines

What is DEEP-1

DEEP-1 is a multi-centre, oral single dose experimental and modelling study to evaluate the pharmacokinetics of deferiprone in patients aged from 1 month to less than 6 years of age affected by transfusion-dependent haemoglobinopathies.

Study design:

- single dose study (three dose levels)
- two phase study:
  1. experimental phase
  2. modelling phase

Objective: to provide supporting evidence for the dose rationale for the use of deferiprone in children aged less than 6 years.

DEEP-1 participants

Sponsor: Consorzio per le Valutazioni Biologiche e Farmacologiche

Central lab: Leiden Universiteit

Centers authorised to recruit:

- A.O.U. Consorziale Policlinico di Bari
- Azienda Ospedaliera di Padova
- A.O. Ospedali Riuniti "V. Cervello" di Palermo
- A.O. "A. Cardarelli" di Napoli
- Clinica Pediatrica Università - ASL1 di Sassari
- Department of Medical and Publich Health of Ministry of Health, Nicosia (opened on 17/12/2013)

Study contacts

Trial leader:
Laura Mangiarini
lmangiarini@cvbf.net

Study scientific coordinator:
Oscar Della Pasqua
medicines.development@gmail.com
Francesco Bellanti
f.bellanti@lacdr.leidenuniv.nl

Trial coordinating investigator:
Giovanni Carlo Del Vecchio
giovanni.delvecchio@policlinico.ba.it

Drug management:
drugmanagement@deep-project.net

Pharmacovigilance:
pharmacovigilance@deep-project.net

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DEEP-1 EXPERIMENTAL PHASE

The experimental phase foresees the recruitment of 18 evaluable children affected by hereditary haemoglobinopathies, aged between 1 month and less than 6 years with a history of at least 10-12 trasfusions.

The first DEEP-1 patient was recruited in Napoli on January 31st, 2013. On December 10th, 2013 the eighteenth patient successfully completed the study in Sassari.

A.O.U. Consorziale Policlinico di Bari	**3 patients**
Azienda Ospedaliera di Padova	**4 patients**
A.O. Ospedali Riuniti "V. Cervello" di Palermo	**3 patients**
A.O. "A. Cardarelli" di Napoli	**4 patients**
Clinica Pediatrica Università - ASL1 di Sassari	**4 patients**

The DEEP-1 Management Team wishes to congratulate all participants for the excellent work and for the spirit of collaboration demonstrated throughout this first phase of the trial.

Study Timelines

Samples analysis:
by January 15th 2014

Modelling:
by the end of February 2014

Study completion:
expected by March 2014

WHAT'S NEXT
DEEP-1 MODELLING PHASE

The study now will proceed with the analysis at Leiden Universiteit on the samples collected on the first 18 evaluable patients.

Using a nonlinear mixed-effects model set up in Leiden, this data should suffice for an accurate assessment of primary pharmacokinetic parameters and for evaluating the impact of parameters such as age and body weight.

During this phase recruitment of patients will proceed in a controlled manner until the precision of PK parameter estimates for Cl/F and V/F will not exceed 40%. If these criteria are not reached with the first 18 patients, additional subjects will be enrolled in the study in order to allow an improvement in the precision of the above parameters.