DEFERIPRONE EVALUATION IN PAEDIATRICS

The research leading to these results has received funding from the European Union (FP7) under Grant Agreement n° 261483

DEEP-1 STUDY NEWSLETTER
ISSUE 2 - 2014

Dear Investigators and DEEP-1 Study Participants,

The second issue of the DEEP-1 trial ("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 affected by transfusion-dependent haemoglobinopathies") is dedicated to the completion of the study and the announcement of the interim analysis results. On the basis of these data, patients under 6 years of age can started being recruited in the DEEP-2 study.

What is the impact of DEEP-1?

This pharmacokinetic study is aimed at the evaluation of systemic exposure of DFP in the paediatric population aged from 1 month to < 6 years, that is, the paediatric subset for which PK profile of deferiprone is unknown.
This lack of information implies that the even the dosage of deferiprone in this age subset can only be calculated empirically based on adult data.
Thus, before assessing the safety and efficacy of deferiprone within the context of DEEP-2, the DEEP-1 study is aimed at providing the daily dosage most appropriate to ensure both safety and efficacy in young children.

DEEP-1 recruiting centres

Sponsor: Consorzio per Valutazioni Biologiche e Farmacologiche

Central lab: Leiden Universiteit

Recruiting Centres:

- 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

DEEP-1 numbers

Number of recruited patients:	**23**
Number of screened but not randomised patients:	**2**
Number of drop-out patients:	**3**
Number of evaluable patients:	**18**

DEFERIPRONE EVALUATION IN PAEDIATRICS

The research leading to these results has received funding from the European Union (FP7) under Grant Agreement n° 261483

DEEP-1 interim analysis

The plasma samples received from the participating centres have been successfully analysed at the Division of Pharmacology of the Leiden/Amsterdam Centre for Drug Research in The Netherlands. The observed deferiprone concentrations were subsequently used to characterise the population pharmacokinetics in this group of patients. The interim analysis results clearly show that all parameters of interest were identifiable and could be estimated with a precision that falls below the threshold of 40% set by the protocol.

DEEP-1 results

Based on these findings, we can confirm that no additional patients need to be enrolled into the study.
The study can officially be considered as completed.

A dosing regimen of 25 mg/kg t.i.d.(i.e. 75 mg/kg/die) is recommended for children aged from 1 month to<6 years, with the possibility of titration up to 33.3 mg/kg t.i.d. (i.e. 100 mg/kg/die), if necessary.

This is the dosage to be used in children under 6 years of age that can now start to be recruited in the DEEP-2 safety and efficacy trial.

For the first time, we have scientific evidence that the dosage of deferiprone used in adults can provide sufficient exposure to ensure efficacy also in small children.

The DEEP-1 team wishes to congratulate all Investigators and each site's study team for the efforts and excellent work which has led to this result.

Trial leader: Laura Mangiarini
Study scientific coordinator: Oscar Della Pasqua, Francesco Bellanti
Trial coordinating investigator: Giovanni Carlo Del Vecchio
Drug management: Rachele Giuliani, Beatrice Pantaleo
Pharmacovigilance: Mariagrazia Felisi, Rachele Giuliani