



Thalassaemia International Federation
World Congress

The DEEP Project

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THE DEEP CONSORTIUM





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DEEP- Deferiprone Evaluation in Paediatrics

- DEEP is a 4 year Project funded by the European Commission within the 7th Framework Program
- Paediatric population from 1 month to less than 18 years
- DEEP studies are investigator driven and multicentric (European and non European countries)
- Partners: Research Centers, Patients associations, no profit institutions, pharmaceutical industry





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DEEP Primary Objectives

- To update Deferiprone SPC, providing data on
 - ▶ pharmacokinetics
 - ▶ Efficacy
 - ▶ Safety

in children aged between 1 month and less than 18 years

- To provide a new formulation specifically tested for children





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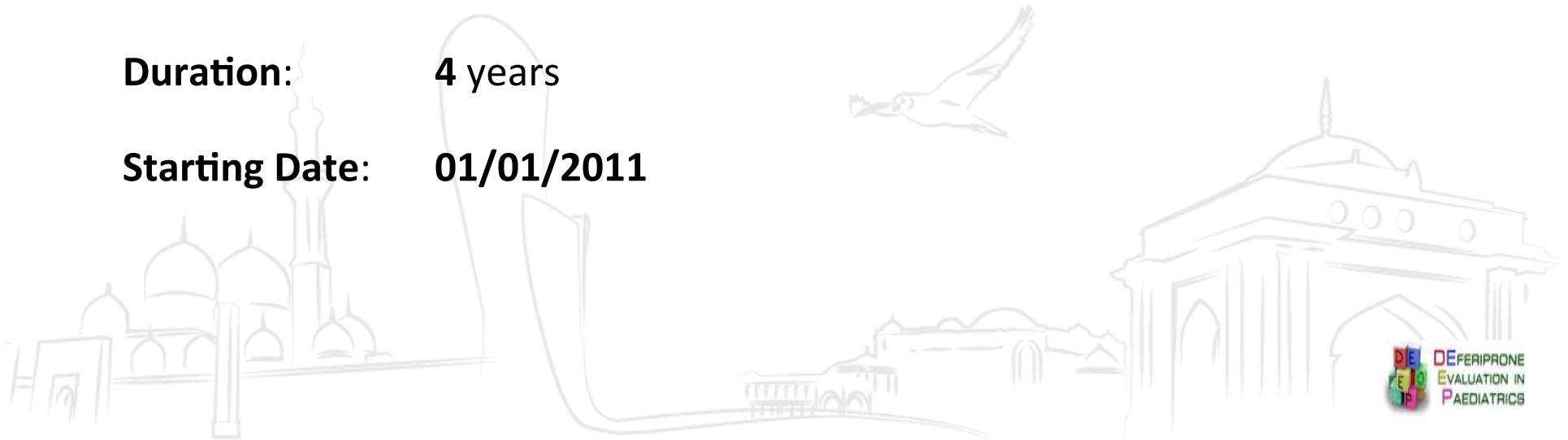
The DEEP PROJECT

Lead Partner: Italy (CVBF- Consorzio per Volutazioni Biologiche e Farmacologiche- Bari)

Partners: 17 Centres

Duration: 4 years

Starting Date: 01/01/2011





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The DEEP PROJECT- Participating Centers

1. Azienda Ospedaliera Ospedali Riuniti Villa Sofia-Cervello, **Palermo - Italy**
2. Cairo University, Cairo - **Egypt**
3. National And Kapodistrian University Of Athens, Athens- **Greece**
4. Qendra Spitalore Universitare "Nene Tereza«, Tirane - **Albania**
5. Department of Medical and Public Health Services of the Ministry of Health, Nicosia - **Cyprus**
6. Azienda Ospedaliera di Rilievo Nazionale "Antonio Cardarelli", **Napoli - Italy**
7. Centre National de Greffe de Moelle Osseuse Tunis, Tunis - **Tunisia**
8. Azienda Ospedaliera di Padova, **Padova - Italy**
9. Azienda Ospedaliero Universitaria Consorziiale Policlinico di Bari, **Bari - Italy**
10. U.O.C. Ematologia - Emoglobinopatie, Azienda Ospedaliera G. Di Cristina, **Palermo - Italy**
11. Presidio Ospedaliero "Annunziata", Centro di Studi della Microcitemia, **Cosenza - Italy**
12. Ospedale Civile di Lentini, Centro di Talassemia, **Lentini (SR) - Italy**
13. Policlinico di Modena, Clinica Pediatrica, **Modena - Italy**
14. Clinica Pediatrica Università - ASL 1 D.H per Talassemia - Pediatria, **Sassari - Italy**
15. Azienda Ospedaliera di Rilievo Nazionale e di Alta Specializzazione Garibaldi, U.O.D. Talassemia, **Catania - Italy**
16. ASL Cagliari Ospedale Regionale per le Microcitemie, **Cagliari - Italy**
17. Azienda Ospedaliera Universitaria Meyer, **Firenze- Italy**





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β -Thalassaemia Major

- Rare Disease: Prevalence in Europe \approx 0.5/10,000
- About 1.5% of the global population is carrier of the disease
- About 200,000 patients worldwide
- Geographical trend: widespread throughout Mediterranean Region, Middle East, Southeast Asia and Indonesia





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β -Thalassaemia Major: The therapeutic approach

1. Frequent **blood transfusions** in order to
 - ▶ suppress ineffective erythropoiesis
 - ▶ to provide adequate oxygen carrying capacity and
 - ▶ to decrease gastrointestinal absorption of iron
2. **Chelation therapy** to remove iron accumulation in tissues (liver, heart and endocrine organs), not otherwise removable through physiological mechanism





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Available Chelators

- **DEFEROXAMINE** (MA 1962) • Treatment of chronic iron overload
- **DEFERIPRONE** (MA 1999) • Treatment of iron overload in patients with thalassaemia major when deferoxamine therapy is CI or inadequate
- **DEFERASIROX** (MA 2006) • Treatment of chronic iron overload due to frequent blood transfusions (≥ 7 ml/kg/month of packed red blood cells) in patients with β -thalassaemia major aged 6 years and older.
 - Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is CI or inadequate in patients aged 2 to 5 years.





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8 Work Packages

WP 1- «Coordination of the Project and General management of the proposed studies across countries»

Specific Objectives:

- To provide the overall scientific strategy and coordination of the project
- To ensure the quality principles of the studies, in accordance with the European Good Clinical Practices and in compliance with the EU Clinical Trials Directive (2001/20/EC)
- To ensure the respect of ethical rules and European and national regulations

WP 2- «Formulation Study- Development of a Paediatric Oral Solution» **ACCOMPLISHED**

Specific Objectives:

- Develop and prepare a drug product suitable for a paediatric population with a small weight range and with appropriate dosage device
- Develop a 80mg/dl oral solution that is more palatable than the one in the market

► *to minimise the risk of non-adherence to therapy* ◀





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8 Work Packages (2)

WP3- «Pharmacokinetic Study»

Specific Objectives:

- to characterise DFP PK after single administration in male and female paediatric patients with hereditary haemoglobinopathies, <6 years of age
- to evaluate the effect of demographic factors on the PK of DFP in children

WP 4- «Paediatric Efficacy/Safety Study»

Specific Objectives:

- compare the efficacy of DFP versus DFX in children affected by hereditary haemoglobinopathies
- to evaluate the treatment efficacy, the safety profiles and the patient compliance

► **START UP: OCTOBER 2013** ◀





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8 Work Packages (3)

WP 5- «Paediatric Long term Safety Study»

Specific Objectives:

- to evaluate long-term safety of Deferiprone use in children by contacting:
 - a **Retrospective cohort study**: will be recruited from existing patients in register database who have received deferiprone before this study
 - a **Prospective cohort study**: patients who receive deferiprone will be prospectively recruited and monitored up to 3 years
- to produce a prescribing guideline which would include the safety data, in order to improve prescribing of deferiprone in paediatric patients

► **Recruitment ongoing** ◀

WP 6- «Pharmacoeconomic Study»

Specific Objectives:

- to evaluate the market and develop a budget impact model (predominantly in European countries)
- to provide an economic evaluation of Deferiprone compared to alternative treatment options

► **Ongoing** ◀





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8 Work Packages (4)

WP 7- «Project Management»

Specific Objectives:

- to provide a management structure adequate to ensure the correct development of the work plan during the project
- to provide resource, procedures and tools aimed to ensure the project proper fulfilment and that all the expected results are delivered on time, within cost and with an adequate quality level

WP 8- «Communication Strategy and the Knowledge Management»

- to ensure efficient communication with stakeholders outside the project in order to raise awareness about activities and results
- to use results of the project for a PUMA application or a Type II variation and for marketing exploitation





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Thank you for your attention

