

AD HOC INFORMATIVE STRATEGIES AS A CRUCIAL TOOL TO EMPOWER CHILDREN AFFECTED BY HAEMOGLOBINOPATHIES INVOLVED IN A MULTINATIONAL CLINICAL STUDY

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BACKGROUND

The need to actively involve children in the decision-making process related to a clinical trial is part of the recently updated guidelines ‘Ethical considerations for clinical trials on medicinal products conducted with minors’ prepared by the European Commission and the Paediatric Committee within the Regulation EU 536/2014.

Despite it is universally established that written communication, combined with verbal interaction, may enhance children’s understanding of their participation in a clinical research, available data and publications show that ad hoc strategies to inform minors to be enrolled in clinical trials are rarely produced.

Careful attention to the contents, format and style of documents addressed to paediatric patients may largely influence their understanding of written documents. For example, the use of pictures, improves the quality of communication, especially for patients with very low literacy skills (Houts et al 2006).

In multi-centre international trials, the difficulty to develop appropriate communication strategies for paediatric patients increases, because of the different cultural and educational backgrounds.

In the framework of the international DEEP project (Deferiprone Evaluation in Paediatrics, Grant Agreement No. 261483), aimed at integrating the existing information on deferiprone use in iron overloaded paediatric patients, an appropriate communication strategy for the paediatric patients involved in the clinical trials has been developed.

AIM AND METHODS

To empower minors in DEEP-2 Study (an efficacy and safety trial to compare deferiprone versus deferasirox in 388 paediatric patients aged 1 month- <18 years from Mediterranean area countries), age-tailored information booklets and assent forms have been prepared. In particular, 3 informative booklets and 2 ad hoc assent forms were prepared for different ages in six languages (Albanian, Arabic, English, French, Greek, Italian) thanks to a collaborative effort involving pharmacologists, paediatricians, child psychologists and illustrators, in an easy-to-manage format, a charming graphic including picture, a simple vocabulary and sentences structures. In order to investigate the quality, the comprehensibility and the likeability of the informative booklets for children involved in the trial, a pilot study (QUBO) has been conducted using age-tailored questionnaires.



CONCLUSION AND FUTURE PERSPECTIVES

This represents a pilot successful experience of appropriate information strategy to paediatric patients involved in clinical trials. In the future, children and families’ active participation in the informative material drafting and evaluation is considered a fundamental step to reach consensus and compliance to treatments.



RESULTS

QuBo pilot study was completed in Albania (at the UHCT and Lushnja centers, in 4 and 9 patients aged 6-10 and 11-18 yrs respectively) and in Italy with 5 patients in Padua centre (3 aged 6-10 and 2 aged 11-18 years) and 3 patients in Palermo centre (aged 11-18 years).

All participants have appreciated the informative booklets in the DEEP-2 trial, both in terms of formatting and style (colours and pictures). Also in terms of understanding, all participants have demonstrated a good comprehension of the key concepts of the disease and the clinical trial (study aim, design and length, administration schedule, possibility to withdraw).

Even if the study was not comparative, it has demonstrated that the adoption of age-appropriate informative instruments is of benefit in paediatric clinical trials.

Consorzio per Valutazioni Biologiche e Farmacologiche - Protocol No: QuBo
Quality evaluation of the informative booklets for patients involved in the DEEP-2 trial (QuBo)

1. QUESTIONNAIRES

5.1 Questionnaire addressed to paediatric patients enrolled in QuBo aged 6-10 years old
ID_CODE: A - - - -

DEEP-2 Deferiprone Evaluation in Paediatrics

Hello boys and girls! Do you want to help us to understand better the Mission Iron Buster booklet?

If you have read the booklet, please answer this short questionnaire. Your answers will help us to get the information we need to be sure the booklet is working and what we can do to improve it. Nobody will give you a mark for your answer and, if you do not want, you can decide at any moment to not answer the questions. Please, write down only your age, not name and surname. You can ask for any question to the doctor that gave you the questionnaire.

Do you want to participate? YES NO

Cross (X) for your answer. Are you ready? Let's GO!

How old are you? ____

1. The booklet was: (please cross one)

a. Easy to read	YES	A little bit	NO
b. Nice	YES	A little bit	NO
c. Boring	YES	A little bit	NO
d. Too long	YES	A little bit	NO

2. Did you like the drawings? (please cross one)

YES	A little bit	NO
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3. Did the drawings help you understand what Dr. Ironless means?

YES	A little bit	NO
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4. Did you learn something new from the booklet about?

a. What are Anaemias?	YES	A little bit	NO
b. Why you need transfusions?	YES	A little bit	NO
c. What medicines take the iron away?	YES	A little bit	NO

5. Red blood cells are in the body and they carry oxygen. (Cross (X) your answer)

TRUE	FALSE
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6. When red blood cells are sick (anaemic) to feel better You have to eat a lot

TRUE	FALSE
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Consorzio per Valutazioni Biologiche e Farmacologiche - Protocol No: QuBo

7. Transfusions bring a whole lot of iron. Medicine helps get rid of iron particles.

TRUE	FALSE
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8. In this research all children will get the syrup.

TRUE	FALSE
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9. Sometimes children taking these medicines might not feel great, but it is normal.

TRUE	FALSE
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10. Children can decide to leave the study when they like and no one will be upset.

TRUE	FALSE
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Thanks for helping!

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Consorzio per Valutazioni Biologiche e Farmacologiche - Protocol No: QuBo
5.1 Questionnaire addressed to paediatric patients enrolled in QuBo aged 11-17 years old
ID_CODE: B - - - -

DEEP-2 Deferiprone Evaluation in Paediatrics

Hi guys and girls! We need your help to evaluate the DEEP-2 MISSION IRONBUSTER booklet. If you have read the booklet, please answer this short questionnaire. Your answers will help us understand how well the booklet works or if we need to improve it. Nobody will give you a mark for your answer and, if you do not want, you can decide at any moment to not answer the questions. Please, write down only your age, not name and surname. You can ask for any question to the doctor that gave you the questionnaire.

Do you want to participate? YES NO

Mark your answer with an X. Thank you for your help!

How old are you? ____

1. What did you think about the booklet? (please mark one for each adjective)

a. Interesting	YES	A little bit	NO
b. Enjoyable	YES	A little bit	NO
c. Too Long	YES	A little bit	NO
d. Boring	YES	A little bit	NO

2. Were there words you didn't understand?

YES, a few	Yes, many	NO
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3. How did you find the content of the booklet? (Please mark one for each question)

a. Easy to understand	YES	A little bit	NO
b. Flat	YES	A little bit	NO
c. Stimulating	YES	A little bit	NO

4. How were the font sizes?

A Just right	YES	A little bit	NO
C Too small	YES	A little bit	NO
D Confusing	YES	A little bit	NO

5. The designs in the booklet were: please mark one for each question

1. Interesting	YES	A little bit	NO
2. Funny	YES	A little bit	NO
3. Useful	YES	A little bit	NO
4. Boring	YES	A little bit	NO

6.1 Did the booklet provide you with useful information about?

YES	A little bit	NO
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6.2 If yes, about?

a. How blood and red blood cells work?	YES	A little bit	NO
b. What transfusion-dependent anaemias are?	YES	A little bit	NO
c. Why iron builds in the body?	YES	A little bit	NO
d. Iron overload treatment?	YES	A little bit	NO
e. The aims of the DEEP-2 trial?	YES	A little bit	NO
f. The different stages of the trial?	YES	A little bit	NO

7. A possible side effect of blood transfusion is iron overload. Antibiotic therapy can remove it.

TRUE	FALSE
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8. The study uses deferiprone and deferasirox drugs to remove iron overload.

TRUE	FALSE
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9. The study needs to deeply study the effects of deferiprone and deferasirox in children and adolescents to ensure they work well.

TRUE	FALSE
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10. Two times per day patients assigned to the deferiprone group take the syrup.

TRUE	FALSE
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11. Who will decide if patients will be assigned to deferiprone or deferasirox group?

Randomly	Physicians	Patients
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12. Can a patient leave the study at anytime?

YES	NO
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13. What is the duration of the treatment?

6 months	12 months	24 months
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Thanks again for helping!

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Reference values

Likeability and acceptance

0-6 : no liking,
7-12: poor liking,
13-18: liking

Comprehension

0-2: no understanding,
3-4: poor understanding,
5-7: full understanding

Likeability and acceptance

0-14 : no liking,
14-28: poor liking,
29-44: liking

Comprehension

0-2: no understanding,
3-4: poor understanding,
5-7: full understanding