



**European Patient Forum on  
Iron Overload in Rare Anaemias**

**October 13<sup>th</sup> 2005**

**Hamburg, Germany**

Participant Meeting Report

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# Meeting Report

## Introduction

The inaugural meeting of the 'European Patient Forum on Iron Overload in Rare Anaemias' was held October 13<sup>th</sup>, 2005 in Hamburg, Germany, with the aims of:

- ◆ Providing participants with an overview on Novartis' research and development activity in the field of iron overload;
- ◆ Improving Novartis' understanding of the needs of European patients with rare anaemia;
- ◆ Providing a forum for the diverse groups representing people with rare anaemias to network and share best practice;
- ◆ Identifying collaborative opportunities for moving forward.

Eighteen representatives from patient groups in Cyprus, France, Germany, Greece, Italy, Switzerland, the UK and the USA attended the meeting (see Appendix 1 for complete list). The thalassaemia, sickle cell (SCD) and myelodysplastic syndrome (MDS) community were all represented.

## Summary

### *Lack of awareness leads to delayed diagnosis and mismanagement of rare anaemias*

There is little clinical awareness about rare anaemias outside of centres of excellence. Most physicians will see one or two cases in the course of their career. Lack of experience, coupled with lack of knowledge, leads to errors of decision-making. Parents' lack of understanding can also lead to delays in diagnosis. Embarrassment resulting from negative attitudes about thalassaemia and SCD exacerbates this situation. Not all patients/parents have sufficient understanding of rare anaemias and iron overload disorder to know when they are receiving a sub-optimal service and to demand appropriate treatment.

Societal prejudices towards immigrant communities can lead to unethical clinical practices. For example, people with SCD going into hospital in crisis are sometimes treated like drug addicts wanting a fix.

There is low awareness amongst physicians about the benefits of chelating therapy for MDS and therefore, iron overload disorder in this patient group is significantly under-treated.

### Current iron overload treatments and monitoring are burdensome

Patients have a “love-hate relationship” with Deferoxamine - they know that the drug is keeping them alive but hate the daily burden of infusions and the impact these have on their quality of life. Infusions are often painful and cause itching. The knot-like swellings caused by the infusions can make it difficult to find injection sites. Experience to date has shown that some patients do not want to take an oral medication three times a day (Deferiprone) and prefer to continue with Deferoxamine infusions.

### There are many emotional and social problems associated with rare anaemias

Given the rarity of the anaemias parents and children often have to travel long distances to obtain adequate treatment. Frequent hospitalisation and hospital visits have a disruptive effect on patients’ and parents’ lives. Everything has to be planned very carefully because of the need for regular transfusions and near-daily infusions of Deferoxamine that means patients are not free to make decisions and live a normal life. It is not always possible to tailor treatments to suit the patient’s life style – this is particularly problematic for adolescent patients. Some parents feel very isolated and left on their own after diagnosis because their doctors show little or no interest in the condition.

People living with rare anaemias often feel different to everyone else because they cannot take part in activities like sport, school outings and social events. The price of acceptance can be to lose something psychologically. Some friends and colleagues are not very supportive because they do not understand the nature of the illness and cannot believe that patients are sick because they do not look sick.

### Compliance with Deferoxamine therapy is a persistent and poorly addressed problem

Compliance with iron chelation treatment is problematic particularly among teenagers. One participant summed up a common attitude held by teenagers: “You either survive and are a nobody or have a good life and are prepared to die by 30 years of age.” Education from doctors and parents on the risks associated with non-compliance has helped improve adherence with treatment. However, parents face a daily struggle to persuade teenagers to comply. A key message for younger patients is that they can live a normal life if they persist with treatment. Patient groups play an important role in promoting this message.

*In general the rare disease community has positive expectations about Deferasirox*

Participants who have had experience with Deferasirox were very enthusiastic about the product. One of the participants was a mother of two children with thalassaemia - one taking Deferoxamine and the other Deferasirox. She was positive about Deferasirox because she has had to spend much less time encouraging her child to adhere to the treatment. Another participant, the father of a child with thalassaemia on a Deferasirox trial, shared his joy about how his child's life had suddenly become 'normal' the day she started taking Deferasirox.

Deferasirox will be welcomed by many patients, as it will do away with the painful and lengthy Deferoxamine infusions and the large number of Deferiprone tablets that need to be taken. However, some have grown very accustomed to Deferoxamine and gain comfort from seeing the drug infuse. These patients may find it difficult to take an oral medication, regardless of its efficacy and side effect profile.

Participants were positive about Deferasirox's side effect profile however, concern was expressed about its gastrointestinal side effects and its effect on creatinine levels. Participants were interested to know whether people with lactose intolerance could take the medication.

Deferasirox will help promote compliance because of its once-daily oral formulation, however, full adherence with treatment cannot be guaranteed because of the myriad factors that contribute to non-compliance, particularly in the chronic disease setting.

It is too early for Deferasirox to have earned the trust of parents and patients. Deferoxamine has been around for forty years and people know it works, despite its burdensome nature. Patients want to know "*What does this new drug guarantee and what can it do for me?*" Even if they trust Deferasirox, some patients will prefer to stick with treatment with Deferoxamine since this is the treatment they are used to and it will be difficult for them to change treatment after so many years.

*The provision of information for patients and parents is sub-optimal*

Significant gaps in the provision of information about iron overload disorder exist and as a consequence, patients and parents often feel that they do not have access to adequate information. This makes it difficult for them to question doctors' decisions or to contribute to treatment decision-making.

Physicians' lack of knowledge about iron overload disorders compromises the quality of patient/parent education, particularly outside of specialist centres. Nurses have an important role to play in meeting patients' and parents'

educational needs, however, few have received adequate education about this condition and consequently, are unable to fulfil this role.

Patient groups have tried to bridge the information gap but their ability to take action in this area is hampered by limited organisational resources. There are examples of best practice (eg. the UKTS have produced a filofax to help patients/parents navigate the thalassaemia journey), however, patient groups have limited opportunities to find out what materials exist in different countries. Language barriers play a role here.

### *Patients/parents access information from a variety of sources*

Doctors are the most important source of information for patients - nurses and patient organisations also play a key role. The Internet is becoming an increasingly important source of information, particularly for the net-savvy younger generation of patients/parents. The Internet is useful for tracking down other patients, the latest research and information about centres of excellence. For some people the Internet is the fastest and most direct way to communicate and find information. However, there is some ambivalence about the value of the Internet because of quality issues. Some people continue to believe that the best place to find information is at hospitals and treatment centres (e.g. transfusion units). Patients currently taking Deferasirox are an important, and highly trusted, source of information about this product.

### *Cultural differences, myths and misconceptions present challenges to optimising patient education amongst the iron overload patient community*

Rare anaemias such as SCD are more prevalent amongst immigrant communities. Lack of awareness about these conditions can lead to myths and misconceptions (e.g. that SCD is transmitted by witchcraft) that can be very difficult to dispel. Language and cultural differences can further compound this problem. In some instances translators are needed, as parents and children do not speak local languages. Also, informational materials are not always available in different languages. Credible and trustworthy community groups can play an important role in dispelling myths and promoting awareness about the conditions. However, the stigma associated with the disease can cause some patients/parents to feel embarrassed and hide their disease from others. In this situation it can be difficult to dispel myths and clarify misconceptions. Ageism is a problem confronting the MDS patient community. The educational needs of elderly people are sometimes ignored or poorly addressed.

### *It is difficult for children with rare anaemias to become independent because of parental overprotection*

Parents of children with rare anaemias are often overprotective of their children because they have a chronic disease. This means that the children

are not given the opportunity to become independent and take age-appropriate responsibility for different aspects of their treatment. The availability of educational materials that are aimed at promoting independence and targeted at different age groups is very limited.

*Novartis can do much to support patient groups representing people with iron overload disorders*

Other than providing sustaining grants, Novartis can help patient groups by:

- ◆ Providing sponsorship for patient representatives to attend conferences and seminars
- ◆ Supporting the translation of books/brochures for different audiences into different languages
- ◆ Supporting patient group newsletters
- ◆ Printing information materials and flyers for patient conferences
- ◆ Providing networking opportunities by organising regular meetings, conferences and seminars
- ◆ Supporting disease awareness campaigns that can promote more positive attitudes about rare anaemias and raise awareness about some of the difficulties faced by patients (eg. limited blood supplies, discrimination etc)
- ◆ Educating medical professionals about advances in the management of iron overload disorders and the use of iron chelators in clinical practice.

*The establishment of a European network of groups representing people with iron overload disorder would be helpful*

A network of representatives from groups representing people with iron overload disorder from different European countries would be useful to disseminate state-of-the-art knowledge and promote the exchange of ideas and best practice. The European network should include all rare anaemias, so that these rare diseases, collectively, will have more power to persuade and influence governments, pharmaceutical companies and medical staff. A European network could also influence European health policy that impacts on the care and treatment of people with iron overload disorders (eg. blood safety, medicines approval etc). Consideration should be given to the name of the group – it may be best to avoid the term “rare”, as thalassaemia is not rare in Greece, and it makes the diseases sound less important.

## **Appendix 1: Participant List**

### **Delegates**

#### **Cyprus**

Loizou Charalambos, Cyprus Thalassaemia Association

Stelios Minas, Cyprus Thalassaemia Association

#### **France**

Suzy Belcou, Association pour l'Information et la Prevention de la Drépanocytose

Jenny Hippocrate, Association pour l'Information et la Prevention de la Drépanocytose

#### **Germany**

Gerhard Bär, Thalassämie Verein Ulm e V.

Ioannis Chatzis, Thalassämie Verein Ulm e V.

Nieddu Cosima, Thalassämie Verein Ulm e V.

Rosemarie Behling, Diamond-Blackfan-Anämie-Selbshilfegruppe e V. Deutschland

Regine Grosse, Universitaetsklinikum Hamburg-Eppendorf (UKE)

Katerina Nassis-Klaus, Thalassemia Initiative Hamburg

Manuela Heine, Hamburg

#### **Greece**

Vasileios Dimos, Thalassaemia Association of Thessaloniki

Kostas Papageorgiou, Pan-Hellenic Association for the Protection of Thalassemia Patients

Panagiota Zissimopoulou, Panhellenic Associaton of Thalassemia (PASPAMA)

#### **Italy**

Tommasina Lorno, Associazione Talassemici e Drepanocitici Lombardi

#### **Switzerland**

Candy Heberlein, Stiftung zur Foederung der Knochenmarktransplantation Schweiz

**UK**

John Mozie, Sickle Cell Society

Philip Nortey, Sickle Cell Society

Chris Sotirelis, UK Thalassaemia Society

**US**

Kathy Heptinstall, MDS Foundation

**Speakers**

Daniele Alberti, Novartis Oncology, Italy

Holger Cario, University Children's Hospital, Ulm, Germany

Gritta Janka, Universitaetskinderklinik, Abteilung für Haematologie und  
Onkologie  
Hamburg, Germany

Antonis Kattamis, University of Athens, Greece

**Novartis**

Jason Coull, Switzerland

Krista McKerracher, US

Marialena Konstantinidou, Greece

Nan Oliver, UK

Regina Vasiliou, Cyprus

Susanna Leto, Italy

Toon van Gool, Italy

**Redmond Consulting**

Kathy Redmond, Italy

Waruni Gammanpila-Easton, UK