Singapore – a country about the size of Greater London, with a population of about 4.5 million, is honoured to be selected as the city to host the next Thalassaemia International Conference. This is a momentous event, as the last time a conference of such significance was held in an Asian country was 1999, nearly 10 years ago, in Bangkok, Thailand. At that time, there were still a lot of closet thalassaemics, air travel was a thing of luxury for the businessmen and the rich in Asia, China was still at the beginning of an ‘open door’ policy, and the lifespan for a thalassaemia major patient was about 15, or so we in Asia were told.

Today, thalassaemia is different. Thalassaemia is no longer a childhood disease. Thalassaemia does not mean education is pointless and career is impossible. Thalassaemia does not mean not able to have a family. The rules have changed. What stayed the same are the attitudes.

It is no longer acceptable for society to see thalassaemics as a burden or as second class citizens. We can’t change attitudes overnight. But we hope by having the opportunities to host thalassaemia conferences in Asia, it will help propel the mindsets to a more positive and confident attitude that is evident in most American and European thalassaemia societies.

This conference we are delighted to have many esteemed and respected speakers from all over the world who passionately stand for the cause of thalassaemia. Prof Sir David Weatherall, Dr Beatrice Wonke, Dr Antonio Piga, Dr Alan Cohen, Prof Suthat Fucharoen, just to name a few. We are fortunate to have Prof Ivy Ng, who started Singapore’s National Thalassaemia Registry to lead the Scientific Advisory Board.

We are also fortunate to have a team of experienced parents and patient leaders to put together a parent/patient programme that runs a full 4-day course as opposed to previous years of only 2-days to ensure parent/patient delegates get the most out of this conference.

The Conference is held in Singapore’s “City-within-the-city” – Suntec City (www.suntecsingapore.com), where it’s located within a precinct which forms a unique, self-contained and fully integrated convention city. Only 20-minutes from the airport, it offers direct access to 5200 hotel rooms, 1000 shops, 300 restaurants, a multi screen cinema and a world-class performing arts centre. Facilities are interconnected and easily accessible via air-conditioned tunnels and covered walkways. It’s everything you want within a 15-minute stroll, not mentioning disability friendly facilities and food from around the world (Halal included). There are social programmes planned for delegates in the evenings, and daily sightseeing trips for accompanying visitors.

The climate in Singapore is tropical – hot, humid and rainy. October is a month when it’s in-between the monsoon seasons, where one would experience frequent afternoon and early evening short and sudden thunderstorms. Therefore I would recommend lightweight cotton or linen clothing and open toe sandals.

Last but not least, check out the Conference website at www.thalassaemia2008singapore.org for full conference information.

Hope to see you there.

By Aggie Michael
Dear Members,

Welcome to the January 2008 issue of the UKTS newsletter.

We often hear the claim “that people are our greatest asset”. Unfortunately, this is usually an empty slogan, a sound bite rather than a reality. However I feel that in the case of the UKTS the Dr’s; the nurses; the researchers; the volunteers; the staff and the people involved with thalassaemia are the UKTS greatest asset.

These are the people that have tirelessly and selflessly worked long hours have made donations, no matter how small, of time or money, to help us with research, projects or the purchasing of equipment that in the long run WILL overcome thalassaemia.

When the society started some 30 odd years ago we started by aiming for the stars, our target was “a cure for thalassaemia”. 30 years on we are still aiming for the stars, for that cure, but the journey has also changed. Along the way we have seen issues that needed to be dealt with. Issues that have made life better by giving choice to all concerned to parents and thalassaemias of all age groups.

We now have a roadmap, and by working with the government, with the NHS and with our people we can see the beginnings of a policy that will allow our full potential to be realized.

All of this allows us to aim for Quality as well as Quantity of life and to eventually land on our star, to find that cure.

This year is a conference year and an international conference serves many purposes. It brings together parents and patients, healthcare professionals and researchers from around the world to one central point. It brings friends old and new together; and it provides support to patients, to families and to associations. So in October 8th – 11th 2008 we head east to the beautiful island of Singapore for the 11th International Conference on Thalassaemia & Haemoglobinopathies and the 13th International TIF Conference for Thalassaemia Patients & Parents http://www.thalassaemia2008singapore.org/

As usual we would like your comment on any of the stories in this issue or if you have any comment to do with thalassaemia in general then email us office@ukts.org.

Until the next issue,

Mike Michael
President
UK Thalassaemia Society
**NHS Sickle Cell and Thalassaemia Screening Programme – Antenatal Screening Not Meeting 3-month Target**

As many of our readers will probably be aware, since 2000 the NHS has implemented an antenatal screening programme for sickle cell and thalassaemia and a newborn screening programme for sickle cell. (Although directed towards identifying sickle cell babies, the newborn screening usually picks up thalassaemias. It also identifies sickle cell carriers, but not thalassaemia carriers.) In the antenatal phase, all pregnant women are offered screening (by a simple blood test) which can tell whether they are carriers of either disorder and, if the test proves positive, the baby’s father is also offered a test. If he too is positive (meaning that the baby has a 25% risk of being born with a major disorder) they are offered counselling and a diagnostic test which will tell them whether or not the baby is affected. Should this be the case, the parents are offered the option of whether they wish to proceed with the pregnancy. It is important to stress that in every case the parents themselves make their own choice on whether they wish to proceed with each stage of testing, and the aim of the programme is to give them as much information as possible about their own carrier status and the health of the expected baby.

Obviously the testing process can be worrying for the parents and can involve extremely difficult personal choices. Given that this is the case, the earlier in pregnancy the tests are carried out, the better. In an ideal scenario, a pregnant woman would be tested before she reaches 10 weeks; however, experience has shown that the ideal scenario is all too rarely found. A recent article by Dr Elizabeth Dormandy and colleagues (British Journal of General Practice, March 2008) describes a study they carried out to determine whether screening was being offered in a timely fashion. In a study involving over 1,400 pregnant women they discovered that only 5% were screened before the target time of 10 weeks, the majority being screened at some time during the second trimester (from 13-24 weeks). In real terms, screening this late in pregnancy offers the couple little opportunity of exercising a choice of whether to proceed should the baby be affected by a major disorder.

In the past there has been speculation that delay in screening is caused in many cases by the fact that women wait too long before reporting their pregnancies to primary care (their GP practice). What this study shows is that, on the contrary, 74% of women reported their pregnancies before 10 weeks; however the average delay from the woman reporting her pregnancy to being screened was a staggering seven weeks.

This study suggests that considerable improvements need to be made in the delivery of the screening process, which will undoubtedly be a challenge. Says Dr Allison Streetly, Programme Director of the NHS Sickle Cell & Thalassaemia Screening Programme; “One solution would be to offer antenatal sickle cell and thalassaemia screening in primary care when women first report their pregnancy. Given the findings reported here, this method would be likely to achieve earlier testing for many women. Such a change, as well as requiring changes in general practice and midwifery care, would be difficult because antenatal sickle cell and thalassaemia screening could be perceived as somehow different from other aspects of maternity care. An alternative way forward would be to acknowledge that sickle cell and thalassaemia screening could be considered as a test for life rather than an antenatal test. It could be conducted in primary care at any stage, not just when the woman is pregnant. To ensure joined-up care, the carrier test result could be included on the maternity referral form, much as relevant history is now included.”

Seeing thalassaemia screening as a “test for life” as Dr Streetly suggests has long been one of the goals of the UK Thalassaemia Society. For carrier testing to be offered to everyone as a matter of routine in primary care, preferably well before they reach the stage of having children, would ensure that carrier status would be an integral part of each person’s medical records just as, for example, vaccinations are recorded at the present time. We are very encouraged to know that the NHS Sickle Cell & Thalassaemia Screening Programme is currently in the process of arranging a special conference around the subject of preconception testing, to take place later in 2008. We hope that this will be the beginning of a process which will ultimately mean that everyone will be offered screening early in life and therefore have the opportunity to make important choices with the benefit of this information. Nothing can take away the fact that being a couple at risk can mean that difficult, sometimes agonising choices have to be made; but being given this information in advance means that at least it does not come as a terrible, totally unexpected shock to a couple who are already expecting a baby.

Despite the delays which are occurring in the antenatal screening process the advent of the NHS Sickle Cell & Thalassaemia Screening Programme has been a massive step forward; as more people are being been screened than ever before and babies’ lives are being saved and health improved by early diagnosis and treatment. In addition, the training for health professionals instituted by the Programme has ensured that awareness and knowledge about thalassaemia and sickle cell disease has reached unprecedented levels within the National Health Service. Other initiatives such as the National Haemoglobinopathy Registry have been instituted with the aim of improving treatment and outcomes for patients in all areas. Finally the powers-that-be are starting to take these conditions seriously. This gradual raising of awareness, both within the Health Service and the general public, is a positive outcome and we at UKTS will continue to work with the Programme until we reach our goal of screening being offered to all pregnant women by 10 weeks – or ideally, before conception.

By Elaine Miller
A new treatment option – Ferriprox® Oral Solution - is now available to patients suffering from thalassaemia major, a rare genetic disease with only 50 percent of the patients surviving the age of 35 years1. Thalassaemia patients require blood transfusions to survive, which leads to damaging iron accumulation in the tissues, especially the heart. Ferriprox® (deferiprone) is an iron chelator that effectively removes the excess iron from the body caused by blood transfusions. A liquid formulation of Ferriprox® is now available in the UK, Germany, Denmark, Norway and Sweden.

For patients with Thalassaemia Major undergoing regular blood transfusions, removal of iron is essential. The introduction of an Oral Solution gives physicians and patients a greater choice of the treatments available. The new formulation of Ferriprox® will make it easier for both existing and new patients to take their medication, said Dr Farrukh Shah, Consultant Haematologist, Whittington Hospital, London.

In this rare disease, new treatment options are even rarer, said Bo Jesper Hansen, CEO, Swedish Orphan International, a distributor of Ferriprox® in Europe. “The solution is ideal for patients who have difficulty swallowing tablets. It is our hope that this new treatment option will improve the compliance across the patient population and thus not only increase the quality of life for the rare disease patients in need but also reduce complications of the disease,” he continued.

The traditional first-line treatment for thalassaemia-related iron overload is deferoxamine, but patients find the treatment difficult, since it involves subcutaneous infusion for eight hours, five or more nights per week. Furthermore, although deferoxamine prolongs life, about half of all patients still die before the age of 35, about 70% of them due to heart failure from iron accumulation in the myocardium.1. Deferiprone was the first oral iron chelator available to thalassaemia patients. A key study now reports that deferiprone appears to be more effective than deferoxamine in removing iron from the heart 2,3, which may explain the reports of observational studies showing improved survival in patients with thalassaemia major4,5.

Anything that makes it easier for a patient to take their chelation therapy is a great thing, and the announcement of this new formulation will help both physicians and patients alike; especially those who have difficulty with their current treatment, said Michael Michael, President UKTS, TIF Board Member.

About Thalassaemia
Thalassaemia is a rare, genetic disorder that affects the production of red blood cells. This leads to severe anaemia at an early age and patients require chronic blood transfusions to survive. Repeatedly transfusing a patient with blood eventually causes iron overload because of the iron in red blood cells. As there is no natural excretory mechanism for iron, it therefore accumulates in the body. If left untreated, iron overload can lead to severe damage of vital organs, particularly the heart.

About Ferriprox® and Ferriprox® Oral Solution
Ferriprox® is indicated for the treatment of iron overload in patients with thalassaemia major when deferoxamine therapy is contraindicated or inadequate.6. Ferriprox® was first approved in the European Union in 1999. It has a well-established safety profile and has been used clinically in more than 7500 patients in more than 50 countries. Ferriprox® oral solution is bioequivalent to Ferriprox® tablets, shows the same benefit-risk profile and is also dosed three times daily.

Ferriprox® has been developed by ApoPharma Inc., a part of the Apotex Group of companies. The company’s expertise is focused mainly in the area of iron chelation.

References:
Optimising a Prenatal Approach for Thalassaemia Gene Therapy

Dr Michael Antoniou  Dr Simon Waddington

Recently, UKTS took the bold decision to fund a new 3-year research project in the laboratories of Dr Michael Antoniou (King’s College London) and Dr Simon Waddington (University College London). The aim of the project is to develop an animal (mouse) model system to assess the possibility of a prenatal approach for thalassaemia gene therapy. This is what is involved and why we are all very excited about the potential benefits of this line of investigation!

As readers of past articles in the UKTS Thalassaemia Matters magazine may recall, current approaches under development for thalassaemia gene therapy all involve the use of an ex vivo strategy, which consists of a number of stages. Firstly, removal of bone marrow from the patient and purification of stem cells. Secondly, introducing a normal functioning copy of the beta globin gene into the purified bone marrow stem cells using a lentivirus gene delivery vector. While all this is going on, the patient undergoes high dose chemotherapy treatment to destroy their diseased bone marrow (“myeloablation”; as with a regular bone marrow transplant). Finally, the genetically corrected bone marrow stem cells are returned back to the patient.

An ex vivo gene therapy approach of this type, has clearly been shown to be potentially a successful method of treatment in animal models of beta thalassaemia. This led to a Phase I clinical trial being initiated in France using this procedure in 2006. However, an ex vivo approach possesses a number drawbacks, which may limit the scope of its application. This includes:

(a) The need for very expensive and sophisticated laboratory facilities to isolate, grow and genetically correct the patient’s bone marrow stem cells.

(b) It is time consuming and labour intensive.

(c) Myeloablation with high dose chemotherapy carries its own major risks of side effects.

(d) It is envisaged that large numbers of bone marrow stem cells will need to be genetically corrected for long-term benefit to be realised; this may not be achievable through an ex vivo protocol.

(e) Cases of alpha thalassaemia major cannot be treated with this procedure, as this condition manifests itself during pregnancy resulting in death of the foetus before birth.

Going prenatal; a viable alternative?

An alternative method that lacks many of the problems associated with an ex vivo strategy is a prenatal approach; that is, before birth. This relies on the fact that in the developing foetus during pregnancy, blood stem cells are present in the liver and not bone marrow. However, starting at about 26 weeks into pregnancy, gradually the blood stem cells migrate from the liver to the bone marrow. The process of switching location of stem cells and blood production from liver to bone marrow is complete just after birth and stays that way for the rest of a person’s life. This sequence of events raises the intriguing possibility that if blood stem cells in a person with thalassaemia can be treated by gene therapy in the foetal liver, then this will result in life-long cure of their condition as the genetically corrected stem cells in the liver will ultimately relocate to the bone marrow and contribute to normal red blood cell production throughout their entire lifetime.

A prenatal approach is based on the direct introduction of the corrective lentivirus gene delivery vector to the foetal liver. It does not involve isolation of bone marrow stem cells and the sophisticated laboratory facilities that this requires and is therefore quick and relatively straightforward to apply using established microsurgery methods. In addition, no myeloablation with high dose chemotherapy is needed, as large numbers of blood stem cells will be corrected within the liver. Finally, both alpha thalassaemia as well as beta thalassaemia can be treated by this approach.

The new research project funded by UKTS aims to combine the skills of Dr Michael Antoniou (globin gene function and lentivirus gene delivery vector expertise) and Dr Simon Waddington (prenatal gene delivery expert) to assess the possibility of a prenatal gene therapy approach for thalassaemia.

Encouraging background work

A number of major developments have taken place over recent years that strongly suggest to us that prenatal gene therapy can be optimised and made into a successful treatment option.

Again as we’ve discussed before in these pages, in the last 5 years it has been clearly demonstrated that lentivirus gene delivery vectors containing beta globin gene units can successfully ameliorate beta thalassaemia major in mouse models of this disease using an ex vivo approach. Based on work previously funded by UKTS in Dr Antoniou’s laboratory, we have constructed a compact lentiviral vector containing a human beta globin gene unit. Prof Giuliana Ferrari (Telethon Gene Therapy Institute, Milan), has shown that our vector functions as efficiently as previously described more complex versions of this lentivirus system and that it too can completely cure the thalassaemia condition in mouse models and in bone marrow stem cells derived from patients with beta thalassaemia major.

Dr Waddington has shown that genetic modification of stem cells via a prenatal approach, gives rise to life-long correction Continues on page 6
Gene Correction Therapy for β-thalassaemia: Making the Break

The β-thalassaemias are caused by defects (mutations) in and around the β-globin gene, within a stretch of DNA that consists of just under 2000 ‘letters’ of genetic code. In the majority of cases, the β-globin genes have suffered a change to just one of these letters (1). It is sobering to think that the effects of such a small genetic change can be so devastating for patients and have led to so much effort to develop and improve treatments, such as iron chelation and bone marrow transplants. In a similar way, it is frustrating to think that if only these small changes could be reversed, patients could be cured.

In fact, there is new hope that just such an approach, gene correction therapy, is now a realistic goal for the treatment of many inherited disorders including β-thalassaemia. Hope arises from recent advances in methods for making designer proteins (called nucleases) that can cut chromosomes at very specific sites—such as the site of a β-thalassaemia mutation. When used appropriately, such nucleases have been shown to increase the chance of successful gene correction.

With this in mind, and with the support of the UKTS, we are developing gene correction therapy using nucleases that will specifically cut the β-globin gene. This might seem rash, given that the β-globin gene is already damaged in β-thalassaemia patients: how will damaging it further, by introducing a double strand break (DSB) into its DNA duplex, be helpful?

The answer lies in the fact that DSBs can be very efficiently repaired by the cell. In fact repair of DSBs is a normal process in every cell which prevents cells from becoming cancerous. Our project exploits the normal DSB repair process to repair the defect in β-thalassaemia. By introducing a fragment of the normal β-globin gene into cells at the same time as the DSB, cells are able to use this as a ‘template’ to repair both the DSB and the β-thalassaemia mutation. The key for efficient gene correction is therefore to deliver two kinds of DNA: a DNA repair template and DNA that will support the synthesis of a nuclease to cut the β-globin gene.

For single-gene disorders such as β-thalassaemia, where current treatments are seldom curative or risk free, gene therapy has been the subject of much research in the last 20 years or so. What is the difference between conventional approaches to gene therapy and gene correction therapy?

Most approaches to gene therapy leave the patient’s mutated disease gene unchanged. What happens is that a miniaturised unmutated version of the gene (this would be β-globin for gene therapy of β-thalassaemia) is inserted into the patient’s cells, and this usually takes up residence at an unpredictable site in or near any one of the thousands of genes present in every cell, in a random fashion. For some rare diseases this conventional approach to gene therapy has been partially successful (2), although there have also been some very serious side effects in some cases (e.g. some children developed leukaemia). Great efforts are being made to make this form of gene therapy safer and to apply it to β-thalassaemia (3), but in the meantime our project is investigating whether we could bypass these problems and risks altogether by correcting the β-globin gene at

References:
Surbek D et al. Perinatal stem-cell and gene therapy for hemoglobinopathies. Seminars in Fetal & Neonatal Medicine, 2008 Apr 15; [Epub ahead of print]
its normal site in the cell.

What are the expected advantages of gene correction therapy? Because a patient’s own β-globin gene, once corrected, is indistinguishable from a normal β-globin gene, we believe that gene correction will be safer, give better control of haemoglobin production and more sustained, longer-lasting effects than approaches that rely on a miniaturised β-globin gene residing at an unnatural location in the cell (4).

What are the scientific details of the project? The immediate challenge is to construct a β-globin gene-specific nuclease. Although formidable, this task has recently been made possible by the development of collections of zinc fingers, regions of protein that bind to short stretches (3 ‘letters’) of DNA (5). By assembling such fingers appropriately and attaching them to a DNA-cutting protein, zinc finger nucleases (ZFNs) can be made that will cut at a gene-sequence determined by the choice of zinc fingers (6-8). An example of one of the first ZFNs to cut a disease gene (a gene that is mutated in patient’s with an inherited immune deficiency, (9,10)), developed by Sangamo Biosciences Inc., is shown in the diagram. ZFNs designed to cut the β-globin gene will need to be assembled and thoroughly tested for their ability to cut the β-globin gene without affecting other genes. Promising ZFNs must then be delivered with a gene correction template into cells that give rise to red blood cells. Although making the template is relatively trivial, efficient delivery to the appropriate cells represents an important longer term challenge.

So far relatively few ZFNs designed for therapeutic purposes have been described, but many, including β-globin-specific ZFNs, are sure to be under development. The success of Sangamo, who lead the field, has been partly based on access to a proprietary database of zinc fingers. Meanwhile, in efforts to make the development of ZFNs more widely accessible, various experts have set up a zinc finger consortium (www.zincfingers.org), published detailed protocols and made reagents available on a non-profit basis (11,12). In deciding to join what may be a crowded race to develop a β-globin-ZFN, we are mindful of the fact that there may be multiple winners, because a single ZFN is unlikely to be applicable to all cases of β-thalassaemia, which can be caused by mutations in many different parts of the β-globin gene. Through the generous help of the UKTS we are now able to develop a β-globin-ZFN in our lab in London. Our driving force is the ambition that this can then be used for specific gene therapy of β-thalassaemia sufferers.

References:

Prof Irene A.G. Roberts
& Dr Andrew C.G. Porter
Imperial College Faculty of Medicine
It was on the 2nd floor, away from the madding crowd, where parents and patients from Blackburn and Sheffield came for an opportunity to meet fellow friends and learn the latest about thalassaemia.

Dr Kate Ryan opened the event with warm welcome to an almost full room. Mike Michael, President of the UK Thalassaemia Society and Board Member of the Thalassaemia International Federation (TIF), delivered a speech from TIF as the Executive Director - Dr Androulla Eleftheriou was not able to attend this event owing to ill health.

Mary Metcalf, a Transfusion Practitioner from the Manchester Royal Infirmary gave a presentation on Blood Transfusion and Blood Safety. It was an in-depth explanation of how the blood was processed from donation to distribution, but not in a technical way, therefore easy to understand by everyone. Ms Metcalf even gave a brief history of how blood transfusion came about, the whys and hows, safety and little quips on how blood type is related to personalities, why things go wrong, the reasons behind requests for a re-cross match, the policies and methods to minimise risks.

Mrs Rasul, Co-ordinator and Vice Chair of NEBATA gave a brief outline of how the Association came a long way since it inauguration back in 1989 in Blackburn to its current status.

Dr Farrukh Shah, Consultant Haematologist of the Whittington Hospital in London, gave a presentation on the three currently available iron chelators, the different types of patients who have difficulty complying with iron chelation, and suggestions to resolve these difficulties. Dr Shah emphasised the importance of regular check ups by a specialist and Dr Kate Ryan informed all that patients no longer need to travel to London for their annual MRI T2* checks. It is now available in Leeds at the Leeds General Infirmary (validated by Royal Brompton in London), under Dr Greenwood. Dr Shah closed by stressing the importance of the doctor/patient relationship.

Lunch was a scrumptious affair. After lunch, Mr Peter Mount – Patron of NEBATA and Chair person for NHS Trust Central Manchester University Hospital gave a brief outline on the foreseeable changes in hospital structure, and urged patients and parents to sign up to become hospital members, to have a voice in shaping the hospital's future.

For the first time, there was focus on Bone Disease and Vitamin D, where Dr Davies, Consultant in Metabolic and Bone Medicine from the Manchester Royal Infirmary gave a very candid talk about Osteoporosis (brittle bones). He stressed the importance of measuring bone mass regularly for all thalassaemia patients as he felt that perhaps it was due to bone marrow activities that caused the enlarging of bone mass thus weakened the bone structure. He emphasised the need for exposure to 20-30 min of sunlight a day during the summer months to boost the vitamin D.

Educational talks for the day ended with Ms Deborah Blackburn from the Northwest Region Child Health to talk about Screening Programme for Sickle Cell and Thalassaemia.

It was heartening to see everyone leaning forward anxiously trying to capture every word being said.

The organisers left sticky pads and pens on tables to encourage participants who were too shy to ask questions openly to write them down and pass to the chair persons to ask on their behalf, which I found was a good way to get questions answered without feeling embarrassed.

There were Christmas cards, calendars and T-shirts from NEBATA for sale in the hope to raise more funds for the association.

The event closed with raffles for prizes donated by members to help raise more funds for the association.

By Aggie Michael
Many congratulations to UKTS Committee member (and thalassaemia patient) Andy Charalambous, who recently celebrated his 50th birthday. Andy, pictured here with his wife Carolyn, also celebrated his 25th wedding anniversary on 27th May 2008. The happy couple renewed their wedding vows on 24th May in a ceremony held at Halsey Masonic Hall, Cheshunt.

A STOMP ALL NIGHT seventies disco was held on Saturday 10th May at Ios bar and restaurant in Southgate, North London – platform shoes, flared trousers and “night fever” dance moves were much in evidence among the colourful crowd.

This event was organised by UKTS President Mike Michael, his wife Aggie and cousin Mario Constantinou. The proceeds of £1,197 were donated to UKTS. Many thanks to those who bought tickets; and to Maria Gavriel, the proprietor of Ios.
8th May Celebrations in the Philippines

Maria Liza T. Naranjo, M.D.
Associate Haematologist and volunteer,
Thalassemia Centre of the Philippines

On May 10, 2008, we held our 3rd celebration of Thalassemia Day to coincide with the celebration of World Thalassemia Day which falls on May 8. We usually choose the Saturday nearest May 8 due to logistic problems during weekdays. On the 10th, we held a lay forum and motorcade in the morning and after a simple fellowship luncheon, held a dialogue with the Philippine National Red Cross, Philippine Blood Coordinating Council, and Department of Health so that patients could directly air out their concerns and needs regarding blood. On May 7, we conducted a basic lecture on thalassemia for health professionals followed by an interactive session so they could be equipped in suspecting and diagnosing thalassemia. We have done mini lectures since last year and we plan to continue targeting the primary physicians who are mostly the first doctors who encounter anaemic patients. A mobile exhibit is likewise planned, which we plan to display in different hospitals and public places so that more people will come to know about thalassaemia. We have also participated in government radio and TV programs to discuss about the condition. We have likewise coordinated with the thalassemia group in Davao; who held their own celebration on May 10. Hopefully next year, the rest of the provinces will follow.

I guess, after 9 years, though a bit slow and difficult, the Thalassemia Centre is finally seeing some of the fruits of its silent but steadfast labour. In fact, this year, other haematologists are now showing signs of interest in participating in our thalassemia advocacy. Since the Thalassemia Centre has existed with scarcely any manpower and literally self funded, this is good news.... more willing volunteers, means more patients that can be assisted.

Here is a photograph of some of our patients from the Thalassemia Centre of the Philippines. The name of their support group is Balikatang Thalassemia. Many thanks to Dr Naranjo for this article and for her wonderful work with the thalassaemia patients of the Philippines.

Encouraging News from Nepal

Our readers may remember meeting little thalassaemia patient Harimaya Upreti, who lives in the Himalayan village of Pokhara, in issue 109 of TM. Since the plight of Harimaya and the thalassaemia children of Nepal was brought to our attention by aid worker Wendy Pinker, UKTS has donated 5 Desferal pumps to the Nepal Thalassaemia Society. Here we see Harimaya having her first Desferal infusion with one of the new pumps. It is our great privilege to make this too small contribution to our friends in Nepal and we hope they will keep in touch.

Blood Safety Workshop in Sofia, Bulgaria

This meeting took place on 31st May 2008 and was organised by the Thalassaemia International Federation in collaboration with the WHO Regional Office for Europe and the Bulgarian Anti-Thalassaemia Association. One of the presentations was given by UKTS Committee member George Constantinou, pictured here (third from right) with the patients, parents and doctors who attended.
Notice to Parents and Teachers

If you teach a child who has Thalassaemia
— You need to read this leaflet

At UKTS we are well aware that many parents of thalassaemic children experience difficulty in explaining the condition to their child’s teachers or carers. We have therefore designed a new leaflet which gives all the necessary information in an easily accessible format so that parents can give these to the teachers at their child’s school. The leaflets are available to parents FREE of charge from the UKTS office – call us now on 020 8882 0011.

UKTS is grateful to the following for their assistance with this project

- Isabel Adams, Thalassaemia Nurse Specialist, Birmingham Children’s Hospital (BCH)
- Munica Bharwani, parent
- Jane Carrington-Porter, teacher, of James Brindley School based at BCH
- Susan Crawford, Specialist Nurse Haemoglobinopathy, Birmingham Sickle Cell/Thalassaemia Services
- Dr Philip J Darbyshire, Consultant Paediatric Haematologist, BCH
- Pamela Hayes, School Nurse, Swanshurst School, Birmingham
- Nazam Rehman, parent

The UK Thalassaemia Society thanks Jeans for Genes for funding this project

Thank You to our Marathon Runners

The Flora London Marathon took place on 13th April 2008. Despite the wet and chilly conditions our runners did a fantastic job and all five finished the race. The combined total of the funds raised so far is £6,270, a marvellous effort from the runners and all those who sponsored them.

All our runners this year are members of Edmonton Running Club. They are (alphabetical order): Angela Antoniou, Tony Lambrou, Constantine Malekkou, Aggie Minas and Chris Minas.

Thanks to Bank of Cyprus

Once again we thank our kind friends and supporters at the Bank of Cyprus, who donated £650 to UKTS on 6th May 2008. The Bank run a scheme whereby they make donations to charities instead of sending Christmas cards; and, as in previous years, UKTS is a grateful recipient! Here we see UKTS Vice-President Chris Sotirelis accepting the donate from Southgate branch Customer Service Manager Maria Sparsi.
Thank you to Sheniz and Chelsea FC

It is our great good fortune to have supportive friends like Sheniz Osman-Jones, who works in the Matchday Hospitality department at Chelsea FC. The club hold a charity raffle in their hospitality boxes during home matches, and once again Sheniz recommended UKTS to be a beneficiary. The raffle, which was held at the Chelsea v Tottenham Hotspurs match on 12th January 2008, yielded £4,125. Here we see Sheniz with her brother (thalassaemia patient) Remis, presenting the cheque to UKTS Committee members.

UKTS thanks Mrs Wilma Nelson

Mrs Wilma Nelson, Chair of the Millpond Tenants & Residents Association in Rotherhithe, South London, received a £3,000 “Local Heroes” award from the Bank of America for her work in her local community. Under this scheme, recipients of the awards can then donate the money to a charity of their choice. We were delighted and honoured when Mrs Nelson appointed UKTS as her chosen charity. UKTS representatives Menuccia Tassone and Elaine Miller attended the Antisocial Behaviour Conference organised by the Millpond Tenants and Residents Association on 28th February 2008 to receive the cheque from Mrs Nelson.

Party Night in aid of UKTS

20TH SEPTEMBER 2008
7.30 pm – 1.00 am
THE FOX PUB (FUNCTION ROOM)
413 Green Lanes
Palmers Green, London N13 5BS

Buffet, Dancing, Karaoke, Raffle Prizes

TICKETS £10 available from UKTS
No children under 12

This fun event is being organised by thalassaemia patient Tanya Yucel. Tanya attends the North Middlesex Hospital for her thalassaemia treatment and is hoping to bring patients from the various London hospitals together to get to know each other. We hope that our London patients will support this worthwhile event and come along – bringing their friends and families of course – for a great night out. All profits from the event will be donated to UKTS. Contact the UKTS office now and book your tickets 020 8882 0011.
DONATIONS

Our most grateful thanks to all our donors for their generosity.

Mr Alan Coombs £15.00
Mrs B. Dattani £15.00
Emir & Huriye Enver (in memory of Serkan Enver) £900.00
Essex Millenium Lodge £420.00
Mr S. Gandhi £410.00
Mrs K Howells £10.00
Mr & Mrs Kaimakkami £100.00
Mr & Mrs Kkafas £10.00
Mrs M. Menelaou (in memory of Mr Menelaou) £25.00
Mill Hill School (Atkinson House) £2,034.00
Parikiaki £700.00
Mrs Z. Pensa £5.00
Mrs Despo Ptohopoulos £50.00
Mr & Mrs Soni (The Gold Centre) £60.00
Dr R.P. Tahalani £55.00

A new way to support your Society

Eagle-eyed readers will have spotted a new feature on the inside back page of Thalassaemia Matters – a standing order form! We have introduced this feature so that our many generous supporters can pledge their donations on a monthly basis without the trouble of writing cheques and posting them off. All it takes is one final trip to the post box with the completed form.

You will see that there are tick boxes for £2, £5 and £10 per month or you can specify any other amount you choose. Remember, UKTS would not be able to continue its work without voluntary donations – any amount you can spare will be most gratefully received! If you are a UK tax payer don’t forget to tick the gift aid box, which will mean that we receive an additional 28% directly from the tax man, so your donation of £10 is worth £12.82 to UKTS!

Thank you in advance for your generous support.

UKTS Welcomes NEW-MEMBERS

Annually

Mrs Meenakshi Meenakshi
Life
Mr Ahmed Gadit

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Neither the Editorial Committee or the Society accept any responsibility for any inaccuracies or omissions.

The views expressed are not necessarily that of the Society.
Please Support The UK Thalassaemia Society by Making a Monthly Donation

| To the Manager [Name of Your Bank] |
| Address |
| City | Postcode |

Please pay: Bank of Cyprus UK, PO Box 17484, 87 Chase Side, London N14 5WH

For the credit of: UK Thalassaemia Society, Registered Charity No: 275107
Sort Code 30-00-42 Account Number 00593812

| The sum of: £2.00 | £5.00 | £10.00 | Other | £ ___________________ (amount) |
| On the ___________________ (day), ___________________ (month), ___________________ (year) |
| And thereafter every month until further notice and debit my account accordingly. |

Name(s) of account holder(s) to be debited:
Account Number:
Sort Code:

Signed ___________________ Date ___________________
Signed ___________________ Date ___________________

Your Address
Tel Number:
Email address:

I would like tax to be reclaimed on my donation under the Gift Aid Scheme. I am a UK tax payer and pay an amount of income tax and/or capital gains tax at least equal to the tax that can be reclaimed on my donation. Please tick.
YES ☐ NO ☐

Please call 020 8882 0011 if you have any queries. When completed, please return to:
UK Thalassaemia Society, 19 The Broadway, Southgate Circus, London N14 6PH.
We will then send this form on to your bank.
Thank you for your valued support.
# UK Thalassaemia Society, 19 The Broadway, London N14 6PH

Charity Reg No. 275107

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## ALL DETAILS AND INFORMATION WILL BE KEPT ON OUR COMPUTERS AND WILL REMAIN IN THE OFFICE AND WILL NOT BE MADE AVAILABLE TO ANYBODY OUTSIDE OF THE UKTS.

If you however do not wish your details kept on our computers please tick this box □

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### Your Personal Details

<table>
<thead>
<tr>
<th>Personal Details</th>
<th>Contact Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>Title (Mr/Mrs/Miss/Ms/Other):</td>
<td>Telephone:</td>
</tr>
<tr>
<td>First Name(s):</td>
<td>Home:</td>
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<tr>
<td>Surname:</td>
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<tr>
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<tr>
<td>Occupation:</td>
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</tr>
<tr>
<td>Ethnic Origin: (Optional)</td>
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</tbody>
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<td>Ethnic Origin: (Optional)</td>
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</table>

### Membership Required (please tick)

- [ ] ANNUAL (£10.00)  
- [ ] LIFE (£100.00)  

(Please make your cheque payable to U.K.T. Society)

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### If you are a patient or parent of a patient please complete the section below

<table>
<thead>
<tr>
<th>Patient’s Name(s):</th>
<th>Consultant’s Name:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Date of Birth:</td>
<td>Consultant’s Telephone:</td>
</tr>
<tr>
<td>Sex: Male</td>
<td>GP’s Name:</td>
</tr>
<tr>
<td>Female</td>
<td>Address:</td>
</tr>
<tr>
<td>Type of thalassaemia: (e.g. Major, Intermedia, Haemoglobin H etc)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Consultant’s Telephone:</td>
</tr>
<tr>
<td>Hospital where-treated:</td>
<td>GP’s Name:</td>
</tr>
<tr>
<td>Address:</td>
<td>Address:</td>
</tr>
<tr>
<td></td>
<td>Telephone:</td>
</tr>
</tbody>
</table>

### Blood Transfused (please tick)

- [ ] Whole  
- [ ] Washed  
- [ ] Frozen  
- [ ] Filtered

### Chelation (please tick)

- [ ] Desferal  
- [ ] Deferiprone  
- [ ] Desferal & Deferiprone

### OFFICE USE: Date Paid ________________ Receipt No. ________________ Approval Date ________________