UNITED KINGDOM
Thalassaemia SOCIETY
matters
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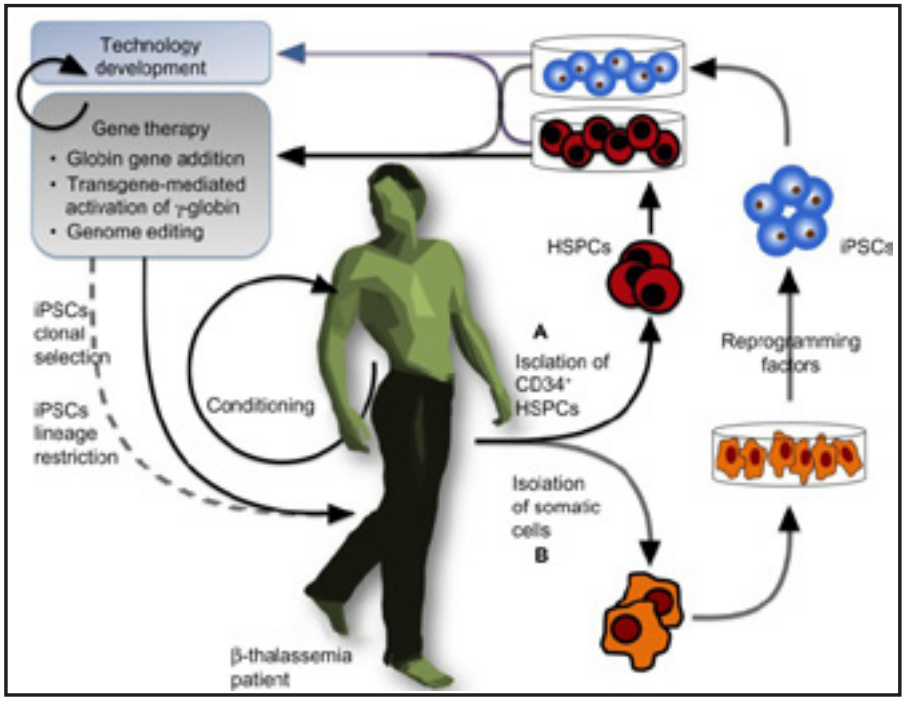
...working for the thalassaemia community

Gene therapy trials begin in the UK

**An update by Dr Perla Eleftheriou,
(Consultant Haematologist University College of London Hospital)**



We have finally and thankfully approached the time when gene therapy can be applied to genetic disorders such as thalassaemia. We can now talk with great optimism about the upcoming phase 3 gene therapy clinical trial in transfusion dependent thalassaemia patients. The phase 3 clinical trial is opening imminently and will play a crucial role in determining whether LentiGlobin has the potential to become a standard of care for carefully selected b thalassaemia patients.

As well known, β-thalassemia is caused by mutations reducing or abrogating β-globin expression, which lead to reduced adult haemoglobin and excess α-globin content in red cells, resulting in ineffective erythropoiesis and early red cell destruction. Most β-thalassemia patients therefore require lifelong clinical management by blood transfusion and iron chelation therapy,with a few having the option of curative but potentially hazardous allogeneic stem cell transplantation. Understandably, this procedure is not suitable for every patient and is generally only offered to children with matched sibling donors, who account for around a quarter of all beta thalassaemia cases. This indicates the need for alternative therapies, which if not to cure the disease, then to reduce transfusion requirements and the significant cost of disease management.

Recent progress in the research of disease modifiers,chemical modulation of gene expression and tools and approaches for DNA-based therapies have opened new avenues toward novel and more personalized strategies to manage or cure β-thalassemia.

In the early phase gene therapy trials, patients had haematopoietic stem cells collected from their bone marrow, which were then transfected with an engineered lentivirus carrying a functional copy of

Continues on page 4 ➡

CONTENTS

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A word from our President

Dear friends,

As some of you may have noticed, a long time has gone by since our last newsletter was circulated. Apologies for the long wait – I hope you will understand that we have been facing an unprecendented workload with very tight deadlines and we have been forced to prioritise – so although we may have been quiet we have had our heads down working harder than ever!

The New Year started on a very sad note for us with the loss of our long-standing Patron and benefactor, George Michael (see page opposite). Since his untimely death it has become evident that George's philanthropic activities were far greater than anyone suspected – unlike many he did not use his charitable giving to add to his own prestige. We, like the other charities George supported, feel his loss deeply and the world has lost a truly exceptional man. Rest in peace George, you will always have a special place in our hearts.

On a lighter note, I would like to thank two very special lady members – Koula Kanias and Tanya Yucel – who both organised fantastic party events to raise funds for UKTS (see page 9). The ladies went to an amazing amount of trouble and truly proved their skill as party planners – Koula and Tanya, you did a wonderful job and on behalf of us all I want to say "Thank you and well done!"

The front page of this issue explains the gene therapy trials which are starting at UCLH – surely one of the most exciting research developments we have seen in many years. Of course, the trials are still at a very early stage, but we will be watching and will bring you more news when it is available.

Just before I go, next year (2018) will be a landmark year for UKTS – 40 years since we first became a registered charity, which makes us one of the oldest, if not the oldest thalassaemia patient societies in the world! We are hoping to bring you some exciting events to celebrate the anniversary so watch this space! And if anyone has any ideas to contribute on the best way to mark our 40-year "birthday" please contact the UKTS office – we are always happy to hear from you.

Until next time, my very best wishes.

Gabriel Theophanous
President, UK Thalassaemia Society

Our Mission Statement

■ **To be the definitive source of information, education and research for those affected by, or working with thalassaemia.**

The UKTS Management Committee

President Gabriel Theophanous	
Vice-President Romaine Maharaj	*Treasurer* Raj Klair
Secretary George Constantinou	*Assistant Treasurer* Tina Bhagirath
Assistant Secretary Anand Singh Ghattaura	*Committee Member* Pany Garibaldinos

RIP George Michael

25.6.63 – 25.12.16

Having experienced the effects of thalassaemia within his own community, George Michael agreed to become a Patron of the Society. He was our Patron and indeed our greatest donor for 15 years. His support touched the lives of thousands of people affected by the condition in the UK and abroad. His annual donation was given with the condition that it was not to be publicised in any way. His support of our work will be sorely missed.

Says Gabriel Theophanous, UKTS President

This was a very sad Christmas for UKTS. Like millions all over the world, we were shocked by the sudden, tragic passing of our Patron, George Michael. As well as being a brilliant and unique artist, George was the most thoughtful and generous Patron to us. For many years he was the largest individual supporter of UKTS; and there have been times when we would have struggled to continue without his support – for example, when our computer systems failed completely in 2005, George came to our rescue. We would have loved to thank George publicly, but he always steadfastly refused to take any credit for his generosity. Only now that he has left us so tragically young can we say how much we owe to him. On behalf of our President, Board and all our members, our heartfelt condolences to George's family and friends. He will always be remembered, not only for his artistic genius but as the kindest and most generous of men.

Continued from page 1

the gene coding for beta-globin, which is defective in thalassaemia. The cells were then infused back into the patient in the hope they would 'seed' the bone marrow and produce functional red blood cells.

Historically, Lentiviral vectors based on human immunodeficiency virus have been developed for this purpose and had been shown to be effective in curing thalassemia in mouse models. Ongoing efforts have been and are being focused on improving the efficiency of lentiviral vector–mediated gene transfer into stem cells so that the curative potential of gene transfer can be consistently achieved.

The results of the early phase gene therapy trials in thalassaemia, demonstrate the potential for a one-time gene therapy treatment to transform the lives of patients with beta-thalassemia major while avoiding immune complications associated with allogeneic stem cell transplant -indeed a long longed vision.

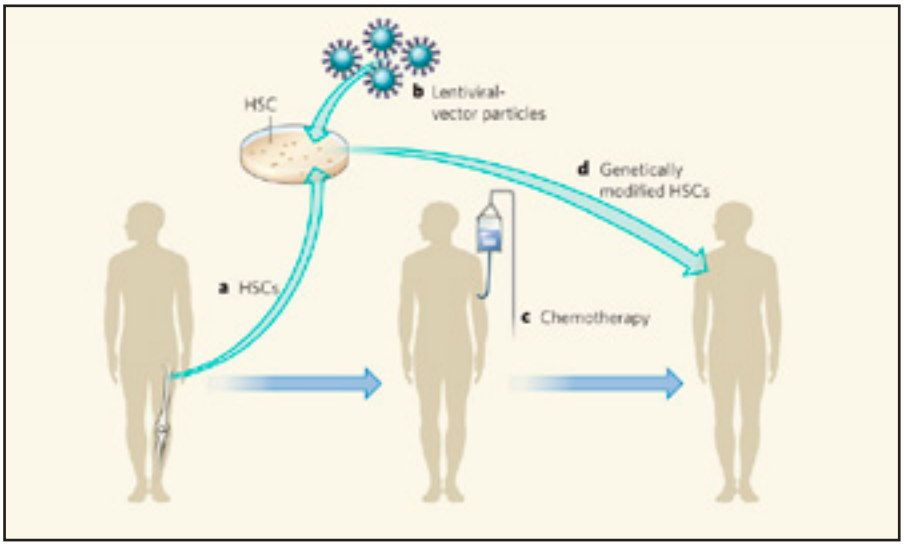
About the early phase gene therapy clinical trials in slightly more detail:

Up until now, 22 patients with thalassaemia and sickle cell disease have had gene therapy within 2 early phase clinical trials run by BluebirdBio.

The Northstar Study is an international, multicenter Phase 1/2 study designed to evaluate the safety in 10 patients with non-β0/β0 genotypes and 8 patients with β0/β0 genotypes who have received LentiGlobin drug product. The median follow-up was 17 months (range: 6.3 – 29.8 months); two patients have completed the two-year primary analysis period.

- All patients with non-β0/β0 geno-types with ≥12 months of follow-up (n=5) have stopped regular transfusions.At last follow-up, the median total hemoglobin of all patients (n=10) with non-β0/ β0 genotypes (median follow up: 14.7 months); was 10.3 g/dL.

- Patients with β0/ β0 genotypes and ≥12 months of follow-up had a median reduction in annualized transfusion volume of 63% and median reduction in annualized transfusion frequency of 65%, calculated based on their transfusion requirements from month 6 to data cut-off. The median follow-up was 17.3 months.



The safety profile remains consistent with myeloablative conditioning using single agent busulfan with no drug product-related adverse events reported.

In the next few weeks we are very excited indeed to welcome a phase 3, multicentre clinical trial by Blue-birdBio, which will open in the UK (UCLH UK site), using an improved manufacturing process that increases transduction efficiency. It is believed that the addition of transduction enhancers to the manufacturing process has the potential to substantially increase the hemoglobin levels in patients with transfusion dependent b-thalassemia and increase their likelihood of achieving clinically meaningful reductions in transfusion requirements or transfusion independence.

The aim of this trial is to evaluate the safety and efficacy of the gene therapy in patients with transfusion dependent b-thalassaemia. The first group of patients studied in this trial will have to have non b0/b0 geno-type but it is hoped that soon the b0/b0 patients will be included in a separate trial. The age group of patients will be 12 and above to 50 or less.The gene therapy will be achieved as in the previous trials, by transplantation of autologous stem cells which were transduced ex vivo (in the laboratory) with the lentiviral b Globin vector. This is an international, multi centre trial and is currently active and enrolling TDT(transfusion dependent thalassaemia) patients in the EU already. The primary efficacy endpoint is the proportion of patients who meet the definition of 'transfusion independence'. This is defined as an average Hb >or equal to 90 g/l without any RBC transfusions, for a continuous period of > or equal to 12 months at any time during the study after drug product infusion. With regard to the way the trial will be conducted: the patients enrolled will first undergo a phase called' mobilisation' whereby stem cells are collected from the blood (apheresis), subsequently they will have to have chemotherapy for 4 consecutive days and finally the stem cells (which had in the meantime been transduced with the b global lentiviral vector) will be re-infused to the patient. As expected, the chemotherapy administered (busulfan), will carry risks of potential complications, such as severe infections, especially whilst the white blood cells remain low. Once the white cell count has recovered and the patient is well, they will be discharged with plans for regular out-patient monitoring the red celland transplant clinics. The follow up within the trial is for 2 years.

We are all (patients and experts) looking forward to following up the progress of the patients who participated in the early phase trials with the particular interest in long term safety. The phase 3 clinical trial is opening imminently and will play a crucial role in the establishment of this curative option as standard of care for carefully selected b thalassaemia patients.

Woman's fertility restored using frozen ovarian tissue

A woman has given birth in London after doctors restored her fertility using frozen ovarian tissue removed when she was a young child.

The 24-year-old is thought to be the first in the world to have a baby after having an ovary frozen before the onset of puberty.

Moaza Al Matrooshi, whose son was delivered at the privately-run Portland Hospital on 13 December 2016, told the BBC: "It's like a miracle.

"We've been waiting so long for this result - a healthy baby."

Her doctor, Sara Matthews, a consultant in gynaecology and fertility, said she was overjoyed for the family - and delighted by the hope it offered to others too.

"This is a huge step forward. We know that ovarian tissue transplantation works for older women, but we've never known if we could take tissue from a child, freeze it and make it work again."

Doctors say it will give hope to many other girls and young women who risk losing the chance of motherhood as a result of treatment for cancer, blood or immune disorders.

Frozen for the future

Moaza Al Matrooshi, who is from Dubai, was born with beta thalassaemia, an inherited blood disorder that is fatal if untreated.

She needed chemotherapy, which damages the ovaries, before receiving a bone marrow transplant from her brother at Great Ormond Street Hospital in London.

So, prior to treatment, when she was nine years old, she had her right ovary removed in an operation in Leeds, where the tissue was frozen.

Fragments of her ovarian tissue were mixed with cryo-protective agents and slowly reduced in temperature to minus 196C, before being stored under liquid nitrogen.

In 2015 surgeons in Denmark transplanted five slivers of the ovarian tissue back into her body - four were stitched on to her failed left ovary and one on to the side of her uterus.

Moaza had been going through the menopause. But after the transplant, her hormone levels began returning to normal, she began ovulating and her fertility was restored.

In order to maximise the chances of having a child, Moaza and her husband Ahmed underwent IVF treatment.

From the eight eggs that were collected, three embryos were produced, two of which were implanted in early 2016.

'Perfect'

Moaza said: "I always believed that I would be a mum and that I would have a baby.

"I didn't stop hoping and now I have this baby - it is a perfect feeling."

She also thanked her mother, whose idea it was to save her young daughter's ovarian tissue so that she might be able to have a family in the future.

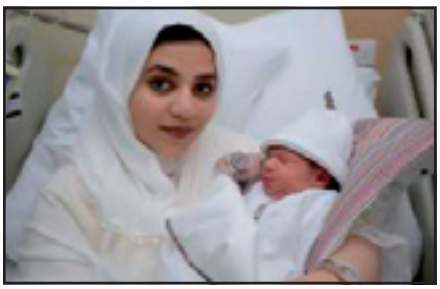
Dr Sara Matthews, who conducted the fertility treatment, said: "Within three months of re-implanting her ovarian tissue, Moaza went from being menopausal to having regular periods again.

"She basically became a normal woman in her 20s with normal ovary function."

Prof Helen Picton, who leads the division of reproduction and early development at the University of Leeds, carried out the ovary freezing.

She told me: "This is incredibly encouraging. Moaza is a pioneer and was one of the first patients we helped back in 2001, before any baby had been born from ovary tissue preservation.

"Worldwide more than 60 babies have been born from women who had their fertility restored, but Moaza is the first case from pre-pubertal freezing and the first from a patient who had treatment for beta thalassaemia."



Moaza al Matrooshi with her baby son

Researchers in Leeds have been at the forefront of ovarian tissue freezing.

In 1999 scientists from Leeds were instrumental in performing the world's first transplant of frozen ovarian tissue.

Prof Picton said that in Europe alone, several thousand girls and young women now had frozen ovarian tissue in storage.

This is usually done prior to patients receiving chemotherapy or radiation treatment, both of which damage fertility.

More babies

Moaza still has one embryo in storage as well as two remaining pieces of ovarian tissue.

She told me she definitely plans to have another baby in the future.

Earlier this year a cancer patient from Edinburgh became the first UK woman to give birth following a transplant of her frozen ovary tissue.

The mother, who conceived naturally, wished to remain anonymous.

Last year a woman in Belgium gave birth using ovarian tissue frozen when she was 13.

Unlike Moaza, she had begun going through puberty when her ovary was removed.

The first woman in the world to give birth following the transplantation of her own ovarian tissue was in Belgium in 2004.

UK Blood Services to Introduce Universal Blood Screening for Hepatitis E

On 1st November 2016 UKTS was invited to a stakeholder meeting to discuss the recommendations of the SaBTO (Advisory Committee on the Safety of Blood, Tissues and Organs) HEV Working Group; which had been reviewing measures aimed at reducing the risk of transmission of Hepatitis E virus via blood, tissues, organs and cells. The stakeholder meeting was held prior to the main SaBTO meeting in the afternoon of 1st November; at which the HEV Working Group introduced their recommendations.

The meeting was chaired by Professor Richard Tedder, who is a member of SaBTO. Prof. Tedder reported on the remit of the HEV Working Group and the background to the investigations. HEV is an RNA virus comprising 4 genotypes. Genotypes 1 and 2 are human viruses and types 3 and 4 are zoonoses (infectious diseases of animals which may infect humans). Types 1 and 2 cause significant health problems in the developing world, being most commonly transmitted via contaminated water. The predominantly affect young adults and cause ~25% mortality in pregnant women. However, genotype 3 is the predominant genotype in the developed economies and transmission is via undercooked infected food, in particular pork or wild boar. Genotype 3 has a low clinical impact; and in healthy individuals it is usually asymptomatic and self resolving. Where symptoms do occur they are low grade and usually non-specific. However, in rare cases neuropathies such as Guillan-Barre syndrome can follow acute infection; and there is evidence that HEV infection can cause decompensation in patients with pre-existing chronic liver disease. Acute or chronic infection may occur in severely immunocompromised patients, e.g. transplant recipients.

In 2015 Public Health England (PHE) received reports of 958 cases of acute hepatitis E of which 791 were acquired in the UK. Investigation based on questionnaires showed an association with processed pork products and HEV infection. The first recorded incidence of HEV transmission by a blood component was in 2006; and since there have been 18 suspected transfusion transmitted cases in England and 5 in Scotland. **Hence it is important to note that the risk of acquiring HEV from diet (see below for dietary advice) is far higher than the risk of acquiring it from blood.** (Genotype 3 is not thought to be sexually transmitted.)

In short, the Working Group had to assess the following facts:

- HEV does not cause significant harm to the vast majority of people; however
- A small group of patients (neonates, severely immunocompromised patients) would be at risk should they acquire HEV (from any source)
- In order to provide only the small at-risk group with HEV screened blood, hospitals and the NHSBT would have to effectively maintain and manage 2 separate stocks of blood. The implications of this are complex and the costs are significant.
- It is necessary to apply the precautionary principle and maintain confidence in the blood supply.

Department of Health health economists used QALY (quality-adjusted life year) and ICER (incremental cost-effectiveness ratios) analyses to model the cost implications of

a) providing the small at-risk group with HEV screened blood and
b) universal screening

Health economics involves extremely complex calculations, but in summary, taking into account staff costs, wastage, the expense of running separate inventories (of both screened and unscreened blood), universal screening is the most cost-effective measure. This is based on the current level of zoonosis in the UK; should this level increase (as has been the case over recent years) the cost effectiveness will increase.

The Working Group therefore recommended that SaBTO issue recommendations to the DH and NHSBT that universal screening for HEV be instituted. Not only is this solution the most practical and cost effective, but it adheres to the ethical medical principle of non-maleficence. In its meeting later that day, SaBTO accepted the recommendations of the Working Group and universal blood screening for HEV will duly be put into operation by UK Blood Services during 2017. Over the last 10 years the incidence of HEV has been rising in all the European countries and is highest in the Netherlands. Thus far no other European countries screen blood for HEV; so the recommendations issued by SaBTO have been received with interest internationally.

Dietary Advice

Our readers may wish to note the following dietary advice relating to HEV is available from the Food Standards Agency (last updated 13.11.14):

Consumers should continue to follow FSA cooking advice which is that all whole cuts of pork, pork products and offal should be thoroughly cooked until steaming hot throughout, the meat is no longer pink and the juices run clear.

One study has suggested heating pork to a core internal temperature of 71°C for 20 minutes is necessary to completely inactivate the virus, however we do not know enough about the levels of hepatitis E virus present in pork more generally to say whether cooking for that long is necessary. Furthermore, cooking under these conditions may not be practical because of the effect on the quality of the meat. There is very little information available on the survival of hepatitis E virus in relation to cooking and not enough evidence to justify a change to FSA advice.

The FSA will shortly be commissioning further research to assess the impact of different time and temperature combinations on the survival of the virus in meat during cooking. The FSA would seek advice from the independent Advisory Committee on the Microbiological Safety of Food before making any changes to our current cooking advice concerning pork and pork products.

UKTS was represented at the HEV Working Group meeting by National Coordinator Elaine Miller

Meet the Trustees

The UKTS AGM was held on 7th September 2016 at the Society's London office. Thank you to all the members who attended; and to Consultant Haematologists Dr Perla Eleftheriou (UCLH) and Dr Emma Drasar (Whittington Hospital) who gave up their time to give presentations and to meet our members.

We would also like to thank outgoing Trustee Pany Garibaldinos for his service over the last few years; and to welcome our new Trustee Tom Koukoulis to the Board.

The Trustees of the Society are (in alphabetical order):

Tina Bhagirath (Assistant Treasurer)

As a career driven British Asian woman with thalassaemia as well as being a single mum, I know the challenges which face us as a community and the stigma of having a disorder. I have had treatment at a handful of hospitals across London before settling at the Whittington. I know the struggles faced by those with thalassaemia in the UK as we try to balance work, home and chelation while trying to establish regular treatment of a certain standard in order to have a good quality of life. I am all about breaking barriers and pushing the boundaries to ensure we have every opportunity to live a full life.

I have been involved in awareness and fundraising work with the UKTS since 2007, and as a member of the committee since 2014; which means I have had the opportunity to take a greater part in the amazing work the UKTS is involved with every day. I have been very fortunate to be involved with the partnership work we do with the NHSBT and TIF as well as organising the May 2016 Fun Run to mark International Thalassaemia Day and promoting the society's participation in the London Marathon for the past three years.

George Constantinou (Secretary)

George is a beta thalassaemia major patient. He is a founder member of the UKTS, having served on the Management Committee from 1976-1985 and again from 1999 to the present day. George has been a tireless campaigner on behalf of thalassaemia all his adult life and has conceived and been involved with many UKTS projects including conferences and awareness projects. He is very involved with the various projects undertaken by UKTS and most notably during the past term, with the production of the 3rd edition of the Clinical Standards and the national patient survey. George is a hotel manager by profession; is married and has a daughter. He has served as Secretary of the Society for several years.

Anand Ghattaura (Assistant Secretary)

Having lived with thalassaemia major for the past 30 years, I know what it is like to have this condition, particularly through school, university, building a career and being treated at a local hospital with limited expertise in thalassaemia. Many people have helped me along the way and this has spurred me to help others and to make a difference. As a life member of UKTS, I have seen the great work the charity has done. After a successful career in the private sector – predominantly in the City of London – I now work for NHS England on a three-year national programme which is implementing new models of care. I am also an active member of the Sikh community in Berkshire.

Raj Klair (Treasurer)

As a 40 year old beta thalassaemia major patient, I understand what it's like to live with the condition and undergo treatment. I want to give something back to society and I believe this is the best way. I own and run a chain of estate agencies in South Yorkshire; and as an entrepreneur I understand the importance of organisation, communication and networking. I believe I can bring these key skills from my professional life to make a valuable contribution to the governance and future development of the Society.

Pany Garibaldinos

Pany is a 59 year old beta thalassaemia major patient. He was a founder member of UKTS in 1976 and served on the Management Committee for many terms in the early years of the Society. He returned in 2010 to add his experience to the Management Committee. As one of the older patients, who has experienced the progression of thalassaemia treatment from the bare minimum of blood transfusions to the sophisticated treatment régimes of the present day, Pany feels that his experiences will enable him to make a valuable contribution to the work of the Society. Pany is married with a son; and he is a driving test examiner by profession.

Romaine Maharaj (Vice President)

Romaine's daughter Roanna is a beta thalassaemia major patient. Prior to moving to the United Kingdom in 2004, she served as the President of the Society of Severe and Inherited Blood Disorders, Trinidad and Tobago, one of the founding members of TIF.

Romaine was also the Head of a mortgage division for one of the major banks in the Caribbean, her financial background spanning a period of twenty three years. Currently she is working as the Student Officer at the Trinidad and Tobago High Commission, London.

Gabriel Theophanous (President)

Gabriel is a 38 year old beta thalassaemia major patient; and has served as President of the Society for the past 5 terms. He works full time in the accounting profession. In 2006 Gabriel became the first person in the world with thalassaemia major to complete a marathon race, a remarkable achievement. In 2013 he repeated this feat when he again completed the London marathon, raising funds for the Society and raising the morale of thalassaemia patients everywhere.

Gabriel and his wife have recently become parents, with the arrival of their twin boys in April 2016.

UKTS thanks our fantastic party planners, Koula and Tanya

A huge **THANK YOU** *to our wonderful members Koula Kanias and Tanya Yucel, who organised fantastic fund raising events for the benefit of the Society. Both ladies have been UKTS members for many years; Koula has been a regular on the dinner dance sub-committee and Tanya is a former Trustee. We are well aware of how much work it takes to organise an event; and we cannot thank Koula and Tanya enough for their magnificent efforts.*

Koula's Event

Koula front right with fellow UKTS members Maria Couppas and friends.

Koula organised a Christmas party, which was held on Saturday the 10th December 2016 at The Venue, Cockfosters. The partygoers danced the night away to the music of DJ Diastello. Koula would like to thank everyone who bought tickets and all the businesses who donated raffle prizes, who are (in alphabetical order) Apollo Restaurant, Andy and Chris Barber, Bel Beauty, Burgeon Flowers, Canon's Fish Shop, Cos of London Jewellers, Dionysus Kebab, Fake It, Gemm Hairdressers, Khin Lee Chinese Restaurant, Lady Fair Hairdressers, Lucia Boxer Osteopathy and The Venue (Luca and Tom). The following also donated contributions to the catering: Aroma Patisserie, Louisa Cakes, Raphael's Patisserie, Wiltons Patisserie.

Says Koula, "It was hard work organising the party but well worth it in the end! I would like to organise more events in future and hope to see more UKTS members coming along to join the fun. I was delighted to be able to donate £2,105 to UKTS from ticket and raffle sales."

Tanya's Event

Night fever at the Fox

Tanya's event took place on Saturday 14th January 2017 at the Fox Pub, Palmers Green. As well as dancing to a DJ the guests enjoyed a buffet supper, live entertainment, raffles and even beauty treatments! And not forgetting a late addition to the programme - a musical tribute to our late, much-loved Patron George Michael, who sadly passed away on Christmas Day 2016.

Says Tanya, "Well after panicking, posting like a crazy woman on Facebook and bugging people to death to purchase tickets, my pestering paid off! The room was bursting with guests who were entertained with Salsa dancers, a salsa class, a lucky dip, mini manicures, neck and shoulder massages and raffle prizes! Everyone`s support was very much appreciated and I would like to thank all who contributed. I raised £2462 and after paying the hire of the hall and the DJ, the society will receive £2092.00!!

UKTS Profile

Dr Emma Drasar

Questions by Elaine Miller, National Coordinator, UK Thalassaemia Society

Dr Emma Drasar has recently been appointed to the post of Consultant Haematologist at the Whittington Hospital, London. Throughout 2016 Dr Drasar has been acting as locum for Dr Farrukh Shah, who has been on a year's sabbatical in Saudi Arabia. During that time, she has endeared herself to the (experienced and demanding!) thalassaemia community at the Whittington by her boundless enthusiasm, caring attitude and meticulous attention to detail. UKTS congratulates Dr Drasar on her appointment and it is our pleasure to introduce her to our readers.

Can you tell us a little bit about your background – where you were born, your education, your family, whatever you wish to share?

Well I was born at UCLH and then lived off the Holloway road until I was 4 years old so this job is a return to my roots for me! I went to school at QE Girls and the Woodhouse 6th form college and then on to The Royal London for medical school. After house jobs and SHO jobs in East London and Kent I then decided to turn to haematology and did my registrar training at King's College Hospital.

I still live south of the river and am married to Tim and have a very lively 8 year old daughter called Beatrice.

At what age did you decide that you wanted to make a career in medicine? Did you choose medicine for any particular reason?

Both my parents are non-clinical (so PhD) doctors although in Bacteriology rather than Haematology. I grew up in the lab – when my Mum's childcare broke down she used to take me into work and the glassware washing ladies used to look after me. Health and safety wouldn't allow that now. I think I had made up my mind to do medicine by the time I was 10 and growing up had my own microscope which I used to use to look at pond water and other things! -

Given that there are so many medical specialities, what attracted you to red cell haematology?

I chose red cell haematology because of the patients. I love the continuity that red cell haematology gives me – really getting to know all about the person not just the symptoms and their diagnosis. The haemoglobinopathies aren't considered glamorous (although many of my patients are) but I wouldn't change my decision for the world. The amount of trust and faith that people put in us is humbling.

Is there any particular person who has been an inspiration to you in your career and if so, why that person?

There have been two people who have been inspirational to me and have really made a difference in my career. The first was my supervisor for my PhD, Prof Swee Lay Thein who previously worked with David Weatherall (in Oxford) and then at King's. She always encouraged me and pushed me to do my best and I would not have had the academic and clinical opportunities I have had without her. The second, and more recent, is Emma Prescott, our thalassaemia goddess at the Whittington. As soon as I stepped onto the unit at the Whittington I could tell that this was a special place and she has taught me an amazing amount in the past year.

What do you see as the main challenges facing doctors treating thalassaemia in the UK today?

One of the main issues is that there aren't enough of us. We know that our senior doctors are moving into a well-deserved retirement but we don't have enough trainees coming through to take their places. It is part of our role as red cell doctors to encourage our juniors into choosing the best sub-speciality!

In your opinion, what significant medical advancements in the treatment of thalassaemia could we see happening in the next 10 years?

This is an exciting time in thalassaemia research. We have gene therapy coming on line, more chelation choice and also novel drugs which might decrease the need for transfusion. However we are also going into the unknown – we have never had thalassaemia major patients in their 60s and our thalassaemia intermedia patients are also getting new complications. Trying to give people quality of life as they age by reducing these complications via good and timely treatment is very important as well. We are building a new evidence base. My area of research has been liver complications in red cell disorders so I want to continue this work in my new role.

What are your favourite hobbies/pastimes when not on duty?

When I have the time I love making cakes and cooking with Beatrice – we make a good team! I also make some of my own clothes (due to my love of bright colours and patterns) and as a family we are keen campers. First camp this year is February half term!

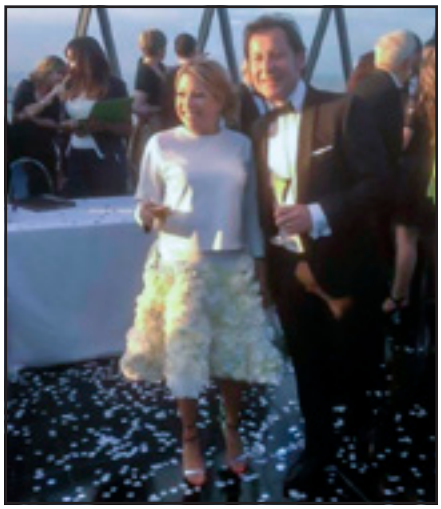
If you had not been a doctor, what would you like to have been?

Probably an archaeologist – probably because of Indiana Jones films though!

Is there anything else at all that you would like to share with us?

I would like to say a massive thank you to all the patients that I have seen in the past year who have made me feel so welcome and the teams at the Whittington and UCLH for their support (Dr Shah via email and skype!).

Congratulations to Tanya and Keith



Our party planning member Tanya Yucel (see page 9) recently celebrated her marriage to her fiance Keith. Weddings are always wonderful, but we think you will all agree that the venue in this case was unusual and truly spectacular – the top of The Gherkin, one of London's tallest and most recognisable buildings. We can always depend on Tanya to put her own unique twist on matters, and this was definitely a one of a kind wedding!

Says Tanya, "Our wedding took place on 7th May 2016 at the top of the Gherkin (more formally known as 30 St Mary Axe), near London Bridge. It was not at all traditional but more of a quirky affair. There was pass the parcel, my top was leather, my shoes had butterflies coming out of them and my cake was decorated with artichokes, cabbages and a field mouse! I wasn't nervous at all, just extremely happy. The views from the venue were spectacular! It was truly the happiest day of my life. If anyone would like to visit The Gherkin, please contact me via The UKTS. The cost is £25 per person which will go to the Society."

Shopping with Salma

– the ultimate in customer service

Personal shopping is until recently used to be the preserve of millionaires and celebrities but this service is now being offered by retailers to all customers – and UKTS member Salma Bi, who works at the Monsoon store in Birmingham Grand Central, is loving her new job helping brides-to-be and businesswomen find the right outfit for every occasion. Says Salma; "I have had customers who have come in with tears of frustration at not being able to find a style to suit their body shape or desire, but who left the store in tears of joy at finding the perfect look."

Salma was born with thalassaemia and underwent a bone marrow transplant at age 13. She is a regular at UKTS events and meetings.

We are always delighted when one of our members sends in a personal experience; but in this issue we have a very special article – from one of our youngest members, Armaan Jameel. Thank you very much to Armaan for his brave and encouraging words and to his Mum Adeeba for sending in the photograph.

My life with thalassaemia major

By Armaan Jameel

Life with thalassaemia, for me has both good and bad points. First I used to find it hard because I had to have blood tests and transfusions with doctors accessing my veins which was painful and there was always a lot of problems. However now I have a Port-a-Cath and my life is much easier because my nurses come home to do the blood tests and I can have cream put on my port which makes it much less painful.

I am a very lucky boy because I love my hospital the nurses are all very caring and my consultant is very nice. Because I go there so often and am the only child with thalassaemia there, they all know me and make sure I am well looked after. Also I am lucky because my teachers can tell when I am feeling low and keep a close eye on me and look after me well at school. It is annoying when sometimes I get tired easily and can't run around too much with my friends.

It's really hard to have thalassaemia since I have to wake up early on school days because I have to have my Exjade and another bad thing is that I have to go to the hospital every three weeks. Also, when I have blood tests and transfusions I have to have needles. But I feel I'm really lucky since I have a port.

I do not get frustrated or jealous that I have thalassaemia and my brothers or friends don't as I was born with it and to me it is just a normal part of my life. I feel lucky that I have so many wonderful people who care for me and look after me wherever I go.

Cardiff event

UKTS National Coordinator Elaine Miller gave an awareness presentation at a patient and family workshop at Butetown Health Centre, Cardiff on 22nd October 2016. The event was organised by the Friends of Cardiff Sickle Cell and Thalassaemia.

Grandmummy

UKTS National Coordinator Elaine Miller started the year with the best possible news – the birth of her first grandchild. Elaine's daughter Grace and son-in-law Ashley welcomed little Evie Grace on 3rd January 2017. Thanks to the staff of the Jessop Wing, Sheffield Teaching Hospitals.

Bank of Cyprus UK

UKTS thanks the Bank of Cyprus

In November 2016 UKTS received a donation of £3,000 from the Bank of Cyprus. This is just the latest example of the Bank's generosity towards the Society; and it is very much appreciated by all of us. On behalf of all our members, our very grateful thanks to the Bank of Cyprus for their continuing support.

UKTS thanks the GIANTS of London

L-R: Nilesh Bathia (seated), Nilesh Raithatha, Kirti Bathia, Katerina Loizi-Read, Mina Raithatha and GIANTS Group current President Kamlesh Rajani

The GIANTS (Generosity, Integrity, Action, Nobility, Truthfulness & Service) Group of London is a small fund raising group that has been supporting the UK Thalassaemia Society since 1988. On February 2017 the GIANTS group presented a donation of £501 to Katerina Loizi-Read, UKTS office administrator. Sincere thanks to all members of the GIANTS group for their valued support; which has been ongoing for the last 30 years.

Introducing the memoirs of Professor Elizabeth Anionwu, who pioneered the role of thalassaemia/sickle cell nurse specialist

'Mixed Blessings from a Cambridge Union' by Elizabeth N Anionwu PhD, CBE, FRCN

Emeritus Professor of Nursing, University of West London

It's 1947 and a clever, sheltered Catholic girl of Liverpool Irish working class heritage is studying Classics at Newnham College, Cambridge. She is the first one in her family to go to university. Mary discovers that she's pregnant. The father is also a student at Cambridge, studying law. And he is black. The fallout from their affair is dramatic, but despite pressure to give up her baby for adoption, the young woman has other ideas. Their daughter Elizabeth grows up to be a Professor of Nursing at the University of West London. This incredible story charts a roller coaster journey from the English Midlands to Nigeria, and from suburban health visiting to political activism and radical nursing. This is a heart-warming and inspiring book about childhood, searching for identity, family, friendship, hope and what makes us who we are.

Mixed Blessings from a Cambridge Union by Elizabeth N Anionwu, is available online from Waterstones and Amazon, price £14.99. Also available as an ebook.

Further information:
www.elizabethanionwu.co.uk

Thalassaemia at the United Nations

UKTS Board member George Constantinou was asked to make a presentation on thalassaemia and the right to health at the 34th Session of the United Nations Human Rights Council, 17 March 2017, Geneva. The medical, public health, economic and social repercussions of thalassaemia and other haemoglobin disorders (as for all genetic disorders), coupled with the global rising of inequality of access to quality healthcare and patient-centred healthcare systems, as defined by the UN Sustainable Development Goals 2030, was discussed by experts of international stature and policy makers at the head of decision-making in countries with high disease prevalence and burden, thus bringing thalassaemia to the forefront of the human rights agenda. Other invited speakers included H.E. the Minister of Health of Cyprus, Dr Georgios Pamporides – representing a country which to-date constitutes a model for the successful control of thalassaemia.

Left to right: ***His Excellency Mr Andreas Ignatiou Cyprus Ambassador to the United Nations, His Excellency Dr Georgios Pamporides Minister of Health for Cyprus, Mr George Constantinou Board Member of the UK Thalassaemia Society and of the Thalassaemia International Federation, Her Excellency Ms Iruthishaam Adam Health Minister for the Maldives, Mr Panos Englezos President of the Thalassaemia International Federation.***

UKTS Scientific Advisory Panel discuss challenging treatment issues

The Scientific Advisory Panel at work

The UK Thalassaemia Society hosted a meeting of the UKTS Scientific Advisory Panel at King's Fund, Cavendish Square, London on 7th March 2017. The objective of the meeting was to discuss specific challenges in treating thalassaemia patients who develop very rare and life-threatening transfusion reactions. Transfusion is such a basic element of thalassaemia treatment that we almost take it for granted, at least here in the UK where we are fortunate enough to have a good supply of safe, screened blood. However, there are very rare circumstances where simply giving a transfusion causes severe and hard-to-treat medical problems; and for these (mercifully) few patients every transfusion, far from being a routine procedure, becomes fraught with danger.

In early February 2017 UKTS was approached by Dr Emma Drasar, Consultant Haematologist at the Whittington Hospital and asked to help in bringing together international experts to discuss not only the causative mechanism but the pros and cons of several treatment options in a particularly challenging case. Further to extensive consultation with Dr Drasar it was decided that we would need eminent doctors from the fields of clinical immunology and stem cell transplantation, as well as those with many years of experience in treating thalassaemia. As the supervising clinician, Dr Drasar felt that the meeting should proceed at the earliest opportunity; the date of 7th March was agreed upon.

It is no mean challenge of itself to bring together a panel of international medical experts with barely a months' notice! Fortunately, however, everyone we approached was incredibly helpful and anxious to contribute. We prepared for the meeting by circulating the patient's medical history (kindly provided by Dr Drasar) and all the panel members were asked to submit any specific queries in advance; for both the medical team and the patient. Hence we were able to have all necessary information and documentation to hand during the meeting. We also provided teleconference facilities for those who were not able to attend in person.

The meeting went ahead very successfully; the doctors present agreeing that getting together around a table and "brainstorming" was extremely productive. The great advantage of this kind of meeting is that it brings together experts from different fields of medicine who would not normally be in the same room and enables them to have a full and frank discussion of all aspects of the case history and treatment options. It was agreed that this same methodology could be used in future to assist doctors struggling with similarly challenging medical problems.

UKTS would ike to thank all those who contributed to the meeting, either as members or as special advisers to the Scientific Advisory Panel.

Chair - Dr Emma Drasar, Consultant Haematologist, Whittington Hospital
Minutes – Elaine Miller National Coordinator, UK Thalassaemia Society
Prof Emanuele Angelucci, Professor of Haematology, U.O. Ematologia Ospedale Oncologico Armando Businco, Cagliari, Italy
Dr Shubha Allard, Consultant Haematologist, NHS Blood and Transplant and Barts Health NHS Trust
Prof Maria Domenica Cappellini, Consultant Haematologist and Professor of Internal Medicine, University of Milan
Dr Josu de la Fuente Consultant Paediatric Haematologist, Clinical Lead for Paediatric Specialties and Director of Blood & Marrow Transplantation, St Mary's Hospital & Imperial College.
Dr Sorena Kiani-Alikhan Consultant in Clinical Immunology, Barts Health & Royal London Hospital
Prof John Porter Professor of Haematology and Consultant Haematologist at the University College London Hospitals in London and Head of the joint Red Cell Unit for UCLH and Whittington Hospitals.
Dr Fernando Tricta Consultant in Paediatric Haematology/Oncology
Dr Paul Telfer Consultant Haematologist, Barts Health & Royal London Hospital
Dr Nay Win Consultant Haematologist and Clinical Director of Diagnostics, NHS Blood and Transplant

Recent Events & Meetings

Those who attended meeting on behalf of the UK Thalassaemia Society are: Gabriel Theophanous *President*, Romaine Maharaj *Vice-President*, George Constantinou *Secretary*, Tina Bhagirath *Asst. Treasurer*, Tom Koukoulis *Trustee*, Monica Tyler *Operations Manager*, Elaine Miller *National Coordinator*, Katerina Loizi-Read *Office Administrator*, Maserat Lal Office *Assistant*

Acronyms

APPG – All Party Parliamentary Group for Sickle Cell & Thalassaemia
CRG – Clinical Reference Group for Haemoglobinopathies
GAUK – Genetic Alliance UK
HCC – Hepatitis C Coalition
NEBATA – North of England Bone Marrow and Thalassaemia Association
NICE – National Institute for Health & Care Excellence
NHSBT – NHS Blood & Transplant
NSC – National Screening Committee
RDMCC – Roald Dahl Marvellous Children's Charity
RDUK – Rare Diseases UK
SCTSP – NHS Sickle Cell & Thalassaemia Screening Programme
SCS – Sickle Cell Society
SHCA – Specialised Health Care Alliance
TIF – Thalassaemia International Federation
UKFHD – UK Forum on Haemoglobin Disorders

- 12 Jan 2016 – meeting with SCS to discuss joint bid for SCT public engagement contract *Romaine Maharaj, George Constantinou, Elaine Miller, Tom Koukoulis*
- 24 Aug 2016 – NICEOffice for Market Access "safe harbour" meeting, London *Elaine Miller*
- 31 Aug 2016 – SCT Public Engagement teleconference *Elaine Miller*
- 7 Sept 2016 – SCT Public Engagement meeting, Guy's Hospital, London *Elaine Miller*
- 7 Sept 2016 – UKTS AGM
- 8 Sept 2016 - Bluebird Bio European Patient Advisory Board meeting, Lisbon *George Constantinou*
- 12 Sept 2016 - UKFHD committee meeting *Elaine Miller*
- 13 Sept 2016 – Yorkshire and Humber Haemoglobinopathy Network meeting, Royal Hallamshire Hospitalm Medical School, Sheffield *Elaine Miller*
- 14 Sept 2016 – University of Bradford Freshers' Fair *Maserat Lal*
- 19 Sept 2016 – Huddersfield community group meeting *Maserat Lal*
- 23 Sept 2016 – Brunel University Freshers' Fair, London *Katerina Loizi-Read*
- 24 Sept 2016 – Bradford Health Mela *Maserat Lal*
- 26 Sept 2016 - SCTSP newborn information governance & clinical advisory group meeting, Guy's Hospital *Elaine Miller*
- 28 Sept 2016 – Freshers' Fair, Tottenham College, London *Katerina Loizi-Read*
- 29/30 Sept 2016 - Middlesex University Freshers' Fair, London *Katerina Loizi-Read*
- 10 Oct 2016 - SCT/PHE Public Engagement teleconference Elaine Miller
- 11 Oct 2016 – APPG AGM, Westminster, London *Monica Tyler*
- 22 Oct 2016 – Sickle Cell & Thalassaemia Workshop, Butetown Community Centre Cardiff *Elaine Miller*
- 24 Oct 2016 - SCT/PHE Public Engagement teleconference *Elaine Miller*
- 1 Nov 2016 – Rare Disease UK meeting *George Constantinou*
- 1 Nov 2016 – SaBTO transfusion stakeholder meeting (HEV), London *Elaine Miller*
- 3 Nov 2016 – West Midlands Paediatric Haemoglobinopathy Network meeting, Wolverhampton *Elaine Miller*
- 8 Nov 2016 – Scottish Medicines Consortium teleconference, *Elaine Miller*
- 11 Nov 2016 – Blood donor selection committee, Skipton House, London *Elaine Miller*
- 11-13 Nov 2016 – TIF Board meeting & Middle Eastern conference, Amman, Jordan *Gabriel Theophanous, Romaine Maharaj, George Constantinou, Raj Klair*
- 15 Nov 2016 – SCT Public Engagement meeting, Guy's Hospital, London *Romaine Maharaj, Monica Tyler, Elaine Miller*
- 29 Nov 2016 – SCT Screening Programme Advisory Group, London *Elaine Miller*
- 1 Dec 2016 – APPG teleconference, *Elaine Miller*
- 9 Dec 2016 – SCT Public Engagement meeting, London *Elaine Miller*
- 12 Dec 2016 – UKFHD committee teleconference *Elaine Miller*
- 19 Dec 2016 – SCT Public Engagement teleconference *Elaine Miller*
- 6 Jan 2017 – SCT Public Engagement teleconference *Elaine Miller*
- 11 Jan 2017 – SCT Public Engagement teleconference *Elaine Miller*
- 18 Jan 2017 – At-risk couples leaflet meeting, London *Elaine Miller*
- 19 Jan 2017 – Meeting Dr Felicity Boardman, London *Monica Tyler, Elaine Miller*
- 23 Jan 2017 – CRG meeting London, Elaine Miller
- 30 Jan 2017 – Blood donor selection committee, Skipton House, London Elaine Miller
- 7 Feb 2017 – CRG teleconference *Elaine Miller*
- 16 Feb 2017 – SCT Public Engagement Advisory Group meeting, Wellington House, London *Elaine Miller*
- 21 Feb 2017 – SCT Public Engagement meeting, Skipton House, London *Elaine Miller*
- 27 Feb 2017 – UKFHD meeting, Guy's Hospital, London *Elaine Miller*
- 6 Mar 2017 – APPG teleconference *Elaine Miller*
- 7 Mar 2017 – UKTS Scientific Advisory Panel meeting, King's Fund, London *Romaine Maharaj, George Constantinou, Elaine Miller*
- 14 Mar 2017 – SCT Public Engagement teleconference *Elaine Miller*
- 17 Mar 2017 – Geneva *George Constantinou*
- 27 Mar 2017 – CRG teleconference *Elaine Miller*

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First Name(s):

Surname:

Address:

Post Code:

Occupation:

Ethnic Origin: *(Optional)*

Contact Details

Telephone: *Home:*

Work:

Mobile:

Fax:

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Are you a:

☐ Patient ☐ Parent/Relative
☐ Healthcare Professional ☐ Association
☐ Other (Please state)

Membership Required *(please tick)*

☐ ANNUAL **(£10.00)** ☐ LIFE **(£100.00)** *(Please make your cheque payable to **U.K.T. Society**)*

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

Patient's Name(s):

Date of Birth:

Sex: ☐ Male ☐ Female

Type of thalassaemia: *(e.g. Major, Intermedia, Haemoglobin H etc)*

Hospital where-treated:

Address:

Consultant's Name:

Consultant's Telephone:

GP's Name:

Address:

Telephone:

Blood Transfused *(please tick)*

☐ Whole ☐ Washed ☐ Frozen ☐ Filtered

Chelation *(please tick)*

☐ Desferal ☐ Deferiprone ☐ Desferal & Deferiprone

Transfusion Frequency:

Units received at each transfusion

Blood Type

OFFICE USE: Date Paid _____ Receipt No._____ Approval Date _____

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