



Professor Marie Scully (MS) – Introduction to the UK TTP Patients Day – Welcome to patients, families and loved ones

TTP – The Patients Journey – Dr Sylvia Benjamin

SB discussed the history of Thrombotic Thrombocytopenic Purpura, discovery of ADAMTS13, and evolution of treatments in TTP. SB shared her journey as a patient with an autoimmune disease, including the uncertainty of living with an autoimmune disease, and understanding the frustration regarding making plans. SB discussed the formation of the South East England TTP registry, and how this enabled growth in research in TTP in the late 90's, and later this extended to become the UK TTP registry. Explained how this has led to more guidelines being developed in the treatment of TTP and how it has increased the evidence we use when making decisions about treatments.

Patient Presentations

Helen – (Message read from Professor Scully) – Detailed Helen's experience with TTP, and returning to her world afterwards. Described how the emotional healing afterwards had been difficult, and how she wished she had taken the support that had been offered. Detailed how discussing her feelings regarding initial delays in diagnosis and treatment had helped her. Thanked medical team for their care.

Jo – Described the process of diagnosis following a sudden stroke. Remembered words such as "VWF" and "ADAMTS13" but could not take these in at the time, but how she felt confident with her care. She found memory issues upsetting, but over the last 2 years she has adapted to this. She detailed how her fatigue had now improved. She saw a counsellor which was if enormous help, and lifted a weight from her shoulders – and found the process of "unburdening" helpful. Thanked medical team for their care.

Zunita – Detailed how her diagnosis was quick and how she was enrolled onto the Hercules trial. On the trial she suffered from some bleeding complications, which she found scary. However, these complications were treated quickly and fully resolved, and she felt better following a blood transfusion. She went home and self-administration of trial injections which she found difficult but managed with some help. Thanked medical team for their care.

MS thanked patients for sharing their experiences and reiterated all patients' journeys are different with different challenges.

Congenital TTP: UK Data - Dr Ferras Alwan

FA explained different disease course in iTTP and cTTP. Explained that in cTTP there are increased numbers of clots formed. Showed research of different rates of clots forming, and also the effect of treating cTTP on the rate of clot formation. Explained that the patients we have studied with cTTP patients may have a normal blood count, but may still have symptoms, including headache, lethargy, fatigue, abdominal pain, but that with treatment these usually improve. Explained we need to treat symptoms seriously to prevent complications.

MS – Reiterated the importance of treatment to prevent long-term complications in cTTP.

Long Term Symptoms in TTP – Dr Tina Dutt

TD explained a growing understanding of the long term consequences of TTP, and the difficulties in returning to everyday life. Explained huge advances have been made with acute TTP. Some of the long-term problems can include high blood pressure, stroke, depression, memory difficulties, concentration, post-traumatic stress disorder and quality of life. Research has shown increased rates





of PTSD and depression following TTP, and unemployment (related to TTP) is a risk factor for this. Treatments can include counselling, pharmacotherapy and medical treatments. We aim to investigate this area as part of the UK TTP registry involving patients.

Commission of TTP Service in England – Professor Marie Scully

MS explained the ongoing specialist commissioning of TTP by NHS England, and how money has been set aside for specialist hospitals and dedicated teams for TTP management. This is a multistage process and has now been approved by the Department of Health. MS explained we do not have control over which hospitals (and how many centres) are approved. The aims of the new model would be to swiftly transfer TTP patients to specialist regional centres for acute care and long term follow-up. This would facilitate a unified approach to treatment, better outcomes, and research in TTP management.

Current Studies and Clinical Trials – Dr Mari Thomas

MT explained three areas of clinical trials;

- i. Replacing ADAMTS13 – currently recombinant (man-made) ADAMTS13 trial now open in cTTP – the hope is we can use recombinant ADAMTS13 in TTP in the future.
- ii. Clearing Antibodies – The elective rituximab study is being used to prevent clinical relapse in iTTP, and is investigating high dose versus low dose rituximab in iTTP patients and is currently open in certain centres in the UK.
- iii. Preventing microvascular thrombi (clots) – Caplacizumab (Hercules Study) showed lower number of plasma exchange was needed and platelet recovery was quicker. It is now available via an early access scheme, and the drug has been included in the national service specification.

Genetic study in Immune TTP – Dr Matthew Stubbs

MJS explained the genetic differences between immune TTP and congenital TTP, and that we still have much to understand around why patients develop iTTP, and why patients have different problems. We are running a genetic study in iTTP trying to find genetic differences in patients with iTTP, in addition to trying to gain understanding as to why TTP patients' diseases behave differently. MJS thanked the UK TTP patients who joined registry and consented to genetic analysis.

TTP Network – Jo McIntyre and Saida Ladha

A tribute was made to Houda Webster for her significant role advancing TTP research and the UK TTP Network.

The UK TTP network is now in its 20th year. It started by printing Jo's story in an article, which subsequently became a newsletter. In 2012 Saida and Houda joined the network. The Networks' aims were to provide links and share information within the field of TTP. Financial support is via the TTP Education and Research Fund. The UK TTP Network support two charities, The TTP Education and Research Fund and Zoe's Wish.

They now have >20 travel translation documents, a FB page (437 members), and a web designer. The website can be visited at ttpnetwork.org.uk, and has around 2000 hits per month, with ~83% being new visits.

MS Thanked all for attendance at the meeting, and for the contributions made.

