The ITP Support Association Platelet Reprint Series

No. 35 – Where we are with ITP

Platelet article reprinted from: **December 2016**

Title: **Where we are with ITP today**

Author: **Professor A.C. Newland**

I would estimate between 800 and 900 adults are diagnosed with ITP in the UK yearly (slightly over 1 per haematologist!). The number of children is probably slightly lower but only 15-20% of those become chronic and most of the rest may not require any treatment.

In the under-60s it remains a more predominant disease in women, as with many other autoimmune diseases. We don't know the reason why – although it is assumed to be related to the hormonal balance. In the over- 60s men become more predominant and this is the age group with the highest incidence.

Treatment always starts with one of the forms of steroids unless they are contraindicated as it is cheap (although not so cheerful!) and the majority of people respond albeit temporarily. There is a lot of current discussion both in the UK and abroad as to whether adding something else in at an early stage can enhance the quality and length of the response and be steroid sparing. A UK study adding in Mycophenolate is being led by Dr Charlotte Bradbury from Bristol University Hospital, and I have also had discussions with Amgen regarding looking at TPOs as a more up front option.

This really merges into the discussion on 2nd line therapy which remains unclear. I published about the decline in splenectomy in the The Platelet last year and there are many discussions on the best 'next-line' treatment depending on the patient's age-group and underlying problems.

We know that the use of splenectomy has dropped off significantly from studying Registry data, as there are now more acknowledged alternatives that are splenectomy sparing. This is being mirrored elsewhere as well. It has, in part, been driven by patient-power as patients realise that while doctors may discuss a 60% success rate, this is really a 40% failure rate and it is this latter group that have most problems. The ITP Support Association has been crucial in highlighting the problems and options.

Because ITP is such a relatively rare disease and no one clinician sees enough to understand outcomes the Registry has been very important in collecting large numbers of patients so we can look at presentation, associated conditions, treatment effects and clinical outcomes. The many publications that have come out of the data is a testament to the importance of such a resource. We now have over 2,000 patients registered.

The enthusiasm that many clinicians have shown in supporting the Registry allowed us to identified those keen individuals who have become the backbone of the ITP Forum. This has allowed us to develop the network of centres in the UK where there is a clinician with an interest in ITP who can help and support their local hospitals. This means more local experience and expertise and should improve the overall standard and quality of care.

The presence of the network means there is a ready body of hospitals available to join in clinical studies so that we can more rapidly look at newer treatments and offer them, where appropriate, to treat difficult cases.

This is an important resource for clinical research looking at genuine areas of need in treatment. Also, by enrolling patients on the Registry and obtaining samples, it allows us to look at some of

the basic underlying questions in ITP, related to cause and the underlying immunological defects, in numbers that provide meaningful results.

At the moment treatment is aimed at treating the effects of the disease and stopping platelets being destroyed or increasing their production, but not at the underlying reasons as to why the disease happened in the first place. There are many units and companies around the world looking at just those questions (or different aspects of them) and a number of agents coming through that may affect the immune imbalance that need studying. The ITP Forum is in an ideal position to take part in those studies and our lab at Barts is one looking at some of the underlying questions.

The ITP Support Association has had a very central role since its inception in providing patient support and education, and stimulating research. Apart from our unit and the work of some individuals there was very little interest in ITP and consequently patients were getting a poor deal. Patients were being treated according to the whims of the local clinician who may not have been up to date and had little access to new treatments and developments. Obviously the internet has changed access but that has only given access to a mass of poorly policed data. The Association has acted as a fulcrum for patient information and education and as a vehicle for putting patients in touch with each other and interested doctors. By supporting both the Registry and the development of the ITP Forum it has helped spread good practice so any patient, wherever they live, can have access to an interested clinician and modern treatment thinking. The post-code lottery of treatment has not disappeared but has been reduced considerably. The stimulation of, and funding for, research has also been a driver in getting patients involved in their disease and the questions they consider important.