

The ITP Forum

www.itpforum.org

Paula Bolton-Maggs
Chair

ITP Forum

- Working group of health care professionals with a special interest in ITP
 - Chair – Paula Bolton-Maggs
 - Secretary – Quentin Hill
 - Treasurer – John Grainger
 - Patient representative – Shirley Watson

Another brilliant idea from Shirley

- Longstanding concerns about management of ITP patients
- Often felt marginalised by the greater needs of people with malignancy
- Many consultant haematologists knew little about ITP
- Treated as a minor and easy to manage disorder
- A need for 'centres of excellence'

STEROIDS!

Good model in haemophilia

- Haemophilia management revolutionised by having centres with experts
- Idea suggested by a woman who invited the interested consultants to tea in Oxford
- Recognised by the Department of Health
- Standard criteria
- Regular peer review
- Establishment of a registry of complications

ITP Forum

- Established in 2011
- Lack of clinical trials
- ITP forum enables discussion among the members of difficult cases
- Two meetings a year
 - Meet in person at the British Society for Haematology annual meetings in April
 - Teleconference every November
- Development of ideas for and support for both
 - Clinical trials
 - research

The ITP Forum

- Objectives:
 - To improve care and outcomes for patients with ITP
 - To provide a forum for interaction of professionals
 - To develop a network of specialist centres
 - To advance the education of health care professionals and the general public
 - To promote best practice
 - To encourage collaborative research and trial recruitment into ITP studies

From haemophilia to ITP centres

- Most consultants managing haemophilia also manage other bleeding disorders
- Including ITP
- National registries
 - Paediatric (Manchester, John Grainger)
 - Adult (The London, Adrian Newland and colleagues)
- Financial support (major) from the ITP support organisation

Criteria for an Adult ITP Clinical Centre

Criteria for a Paediatric ITP Clinical Centre

Centre Directors



National Centres

- Aberdeen Royal Infirmary
- Addenbrookes Hospital, Cambridge
- Royal Belfast Hospital for Sick Children
- Belfast City Hospital
- Birmingham Children's Hospital
- Bristol University Hospitals
- Bristol Royal Hospital for Children
- Children's Hospital for Wales
- Coventry & Warwick University Hospital
- Derriford Hospital, Plymouth
- Edinburgh Royal Hospital for Sick Children
- Glasgow Royal Infirmary
- Kent & Canterbury Hospital
- Leeds Children's Hospital
- Leeds St James's University Hospital
- Leicester Royal Infirmary
- Manchester Royal Infirmary
- Norfolk and Norwich Hospital
- Oxford University Hospital
- Queen Elizabeth Hospital, Birmingham
- Royal Manchester Children's Hospital
- Royal Victoria Infirmary, Newcastle
- Southampton University Hospital
- University of Wales Hospital

London Centres

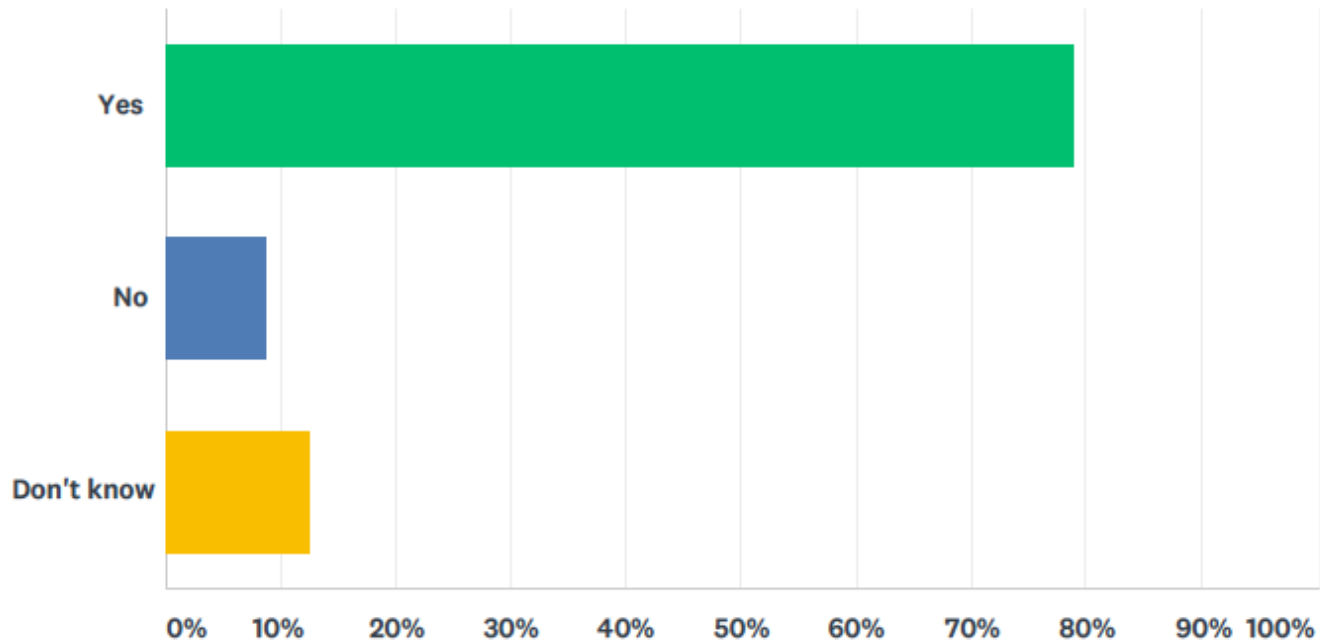
- Great Ormond Street Hospital
- Guy's and St Thomas'
- Evelina Children's Hospital
- Hammersmith Hospital
- Royal London Hospital
- St. George's Hospital
- University College London Hospital

Survey of ITP Centres Autumn 2017

- 245 responses relating to 27 centres
- 98% happy with quality of care
- 9.44 average score out of 10 – first class care

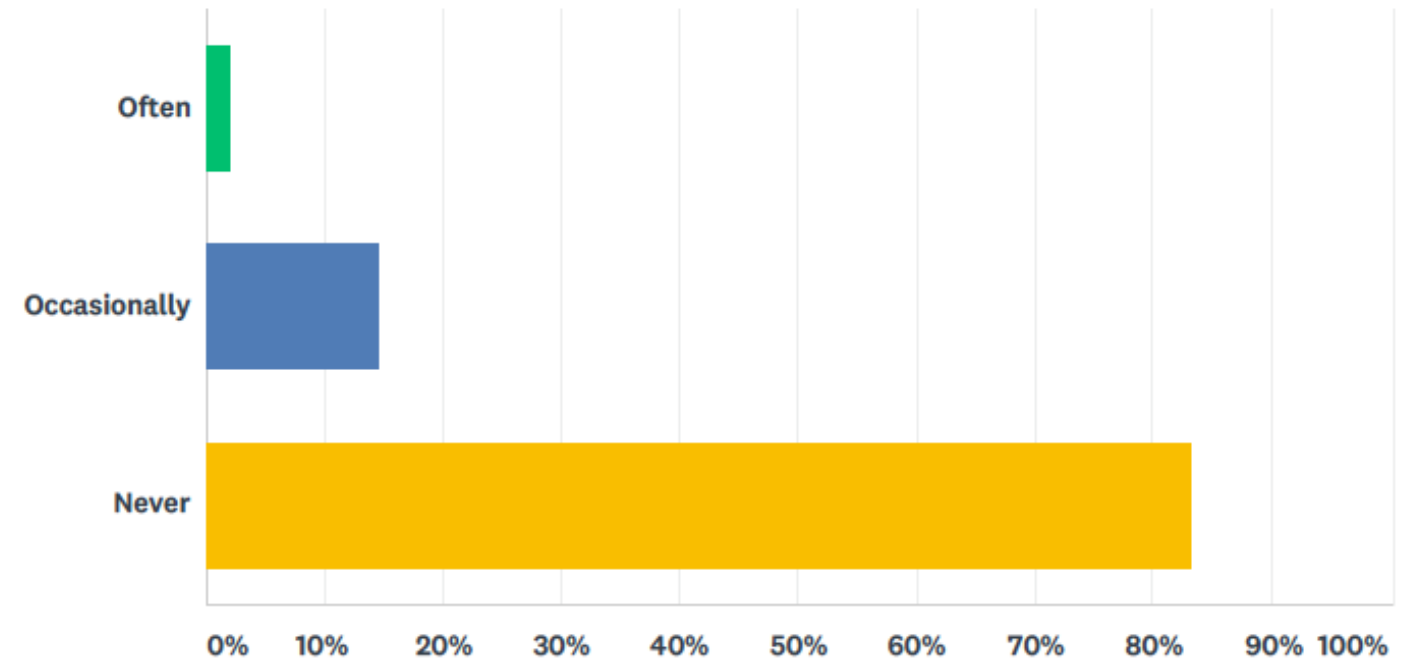
Q6 Does your doctor have a treatment plan for you? (Yes / No / Don't know)

Answered: 241 Skipped: 5



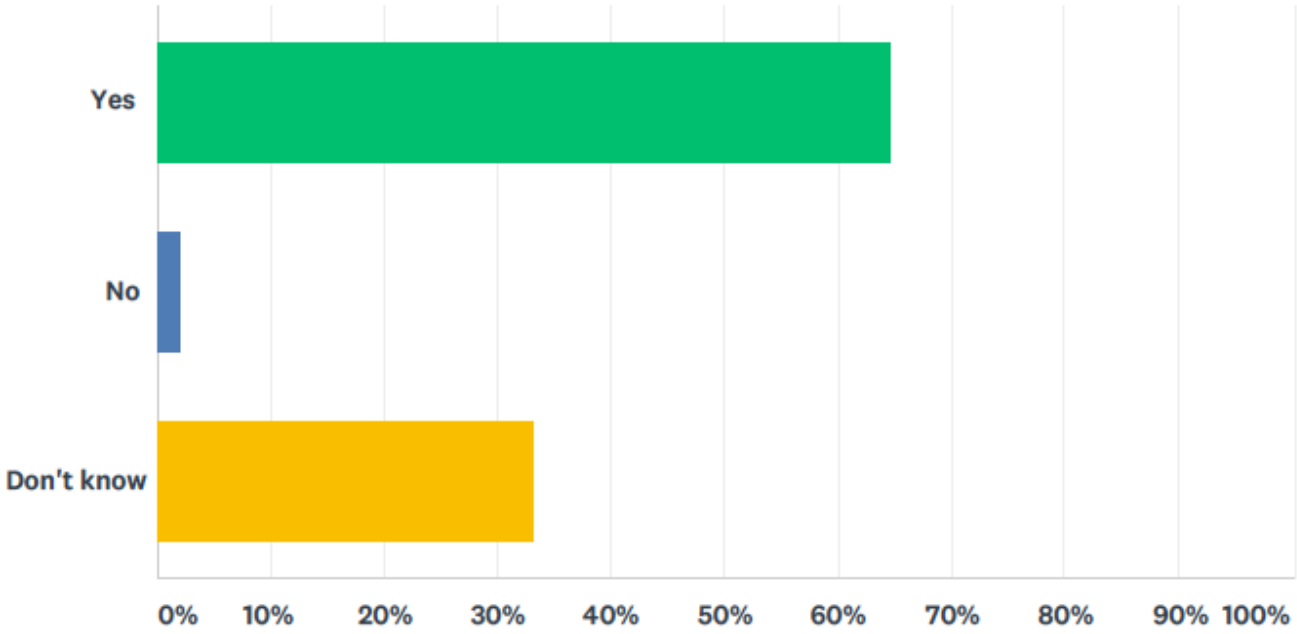
Q7 Have you ever had conflicting advice from different doctors in the team? (Often / Occasionally / Never)

Answered: 245 Skipped: 1



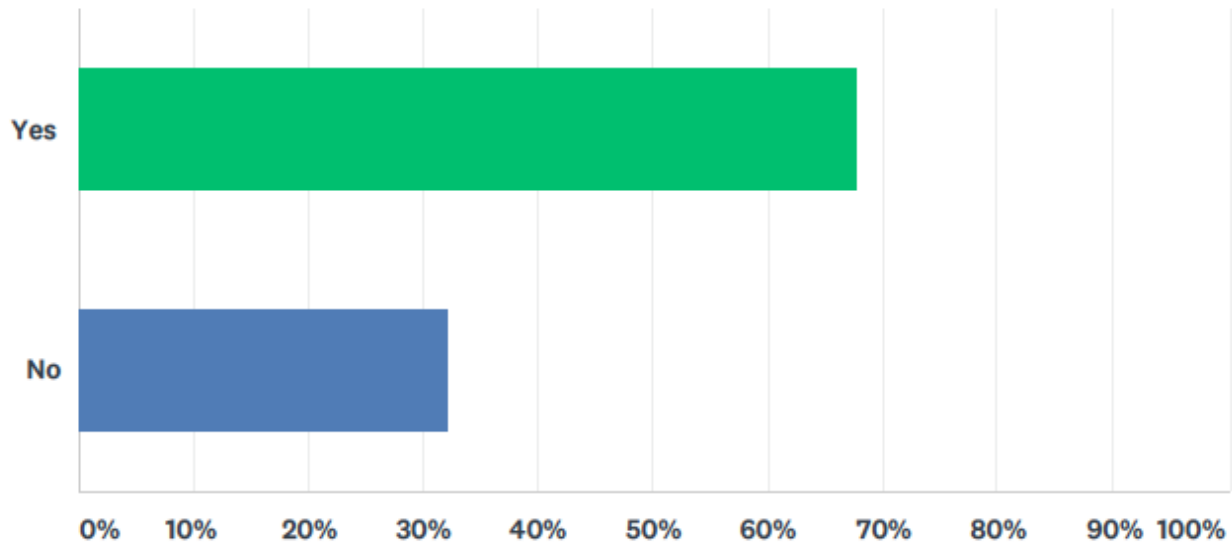
Q8 Is there an ITP specialist nurse in the clinic? (Yes / No / Don't know)

Answered: 241 Skipped: 5



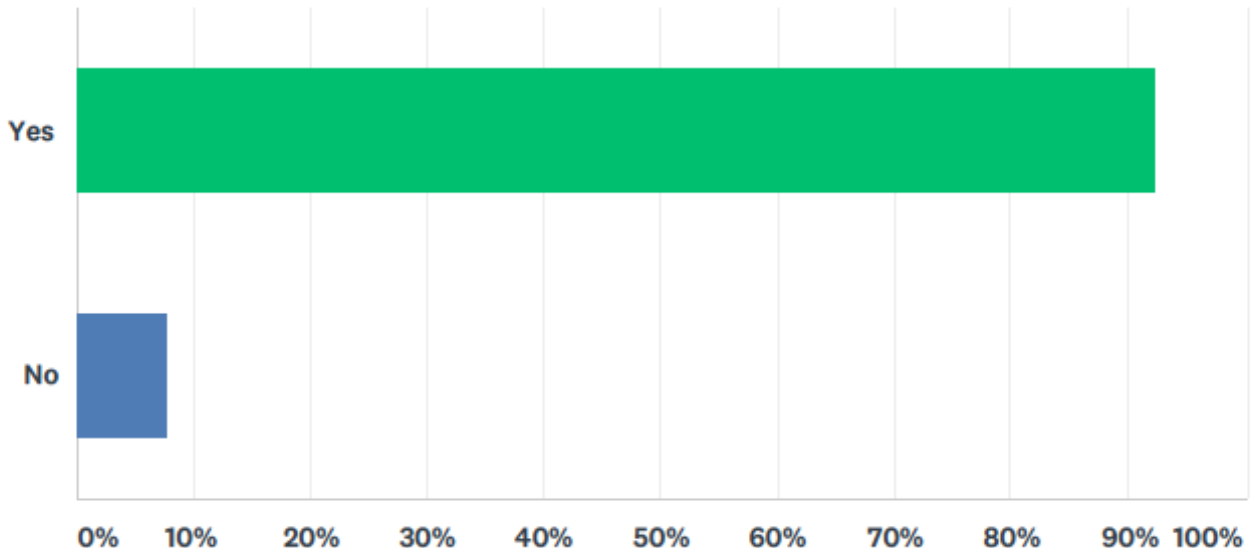
Q17 Have you been offered the opportunity to take part in any clinical trials or studies? (Yes / No)

Answered: 239 Skipped: 7



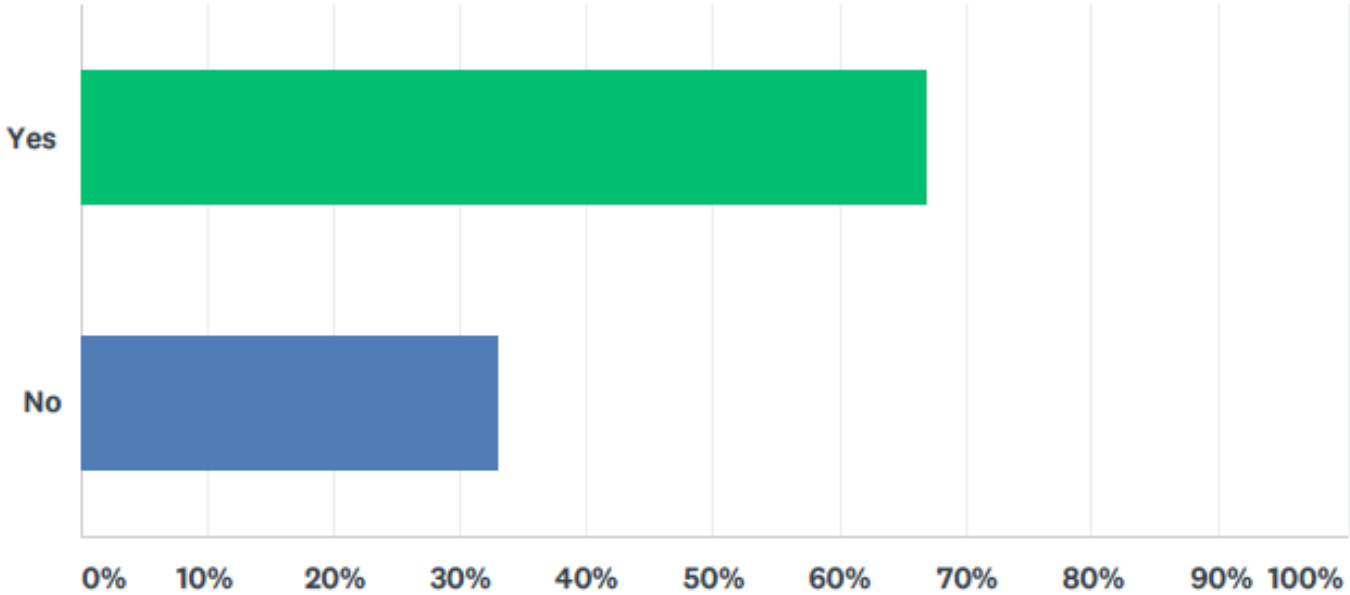
Q18 Have you been given a number to ring in case of emergencies or urgent enquiries? (Yes / No)

Answered: 244 Skipped: 2



Q20 Were you provided with information about the ITP Support Association during your first visit? (Yes / No)

Answered: 239 Skipped: 7



ITP registry for children

- Supported by the association since 2005
- Multicentre study of children with ITP from 2 months to 18 years
- Initial data collection at diagnosis and at 6 months
- Long term follow up every 12 months
- Additional information for children with severe bleeding or bleeds in the head
- Includes quality of life

Paediatric registry (15 years)

Update from John Grainger

- 1694 patients <18 yrs
- 129 centres
- 2014-15 about 100 per year
- 532 in last two years, 67% newly presenting cases in the UK
- Generous donation of £20,000 from a parent for IT update
- Regular presentations at American Society of Hematology meetings
- 3-month projects for medical students
- Various projects planned for 2018/19

Collaborative studies and research

FLIGHT – a multicentre randomised study

- 120 patients from 20 hospitals
- Initial treatment either
 - Mycophenolate plus steroids
 - Steroids alone
- Grant £350K (NIHR grant)
- Start date Aug 2017, end Aug 2010
- Dr Charlotte Bradbury, Bristol

UK ITP forum study

- ITP forum review of practice: TPO agonists
- A 12-question survey: responses from 2/16 paediatric and 14/20 adult ITP centres
- Adult centre responses analysed
 - Most would use TPOs before splenectomy
 - Recommended that these be considered in patients who have failed first line

(Paper in Br J Haematol 2018 is available from link on ITP forum website)

Nichola Cooper's centre, Imperial College London:

Clinical research

- **Understanding the risks of thrombocytopenia**
 - MRI brain study
 - Genomics
- **Phase 1 studies ITP**
 - Staphylococcal protein A
 - Neonatal FcR inhibitor
- **Phase 2/3 studies ITP**
 - Spleen tyrosine kinase inhibitor
 - Romiplostim in children
- **Longitudinal prospective study of treatment outcome**

Basic research

- **Phenotyping** ITP to distinguish subtypes
- Designing a **platelet production model** system to interrogate ITP
- Using trios and complex phenotypes to study the **genomics** of ITP
- Investigating **T and B cell** causes of disease

Studies and Clinical Trials

UCB TP0001: Phase II study; A multicentre, open-label study of TP0001 in adult patients with persistent/chronic ITP; Inclusion criteria: platelet count $\leq 30 \times 10^9/L$. UK principal investigator Dr Nichola Cooper, Imperial College Healthcare Trust, London

PRTX-100 in ITP: Phase 1b study; A multicentre, open-label Study of PRTX-100 in adult patients with persistent/chronic ITP; Inclusion criteria: platelet count $\leq 30 \times 10^9/L$. UK principal investigator Dr Nichola Cooper, Imperial College Healthcare Trust, London

FLIGHT Study: A multicentre randomised trial of first line treatment pathways for newly diagnosed Immune thrombocytopenia to compare standard steroid treatment against combined steroid and mycophenolate <https://www.cardiff.ac.uk/centre-for-trials-research/research/studies-and-trials/view/flight> .

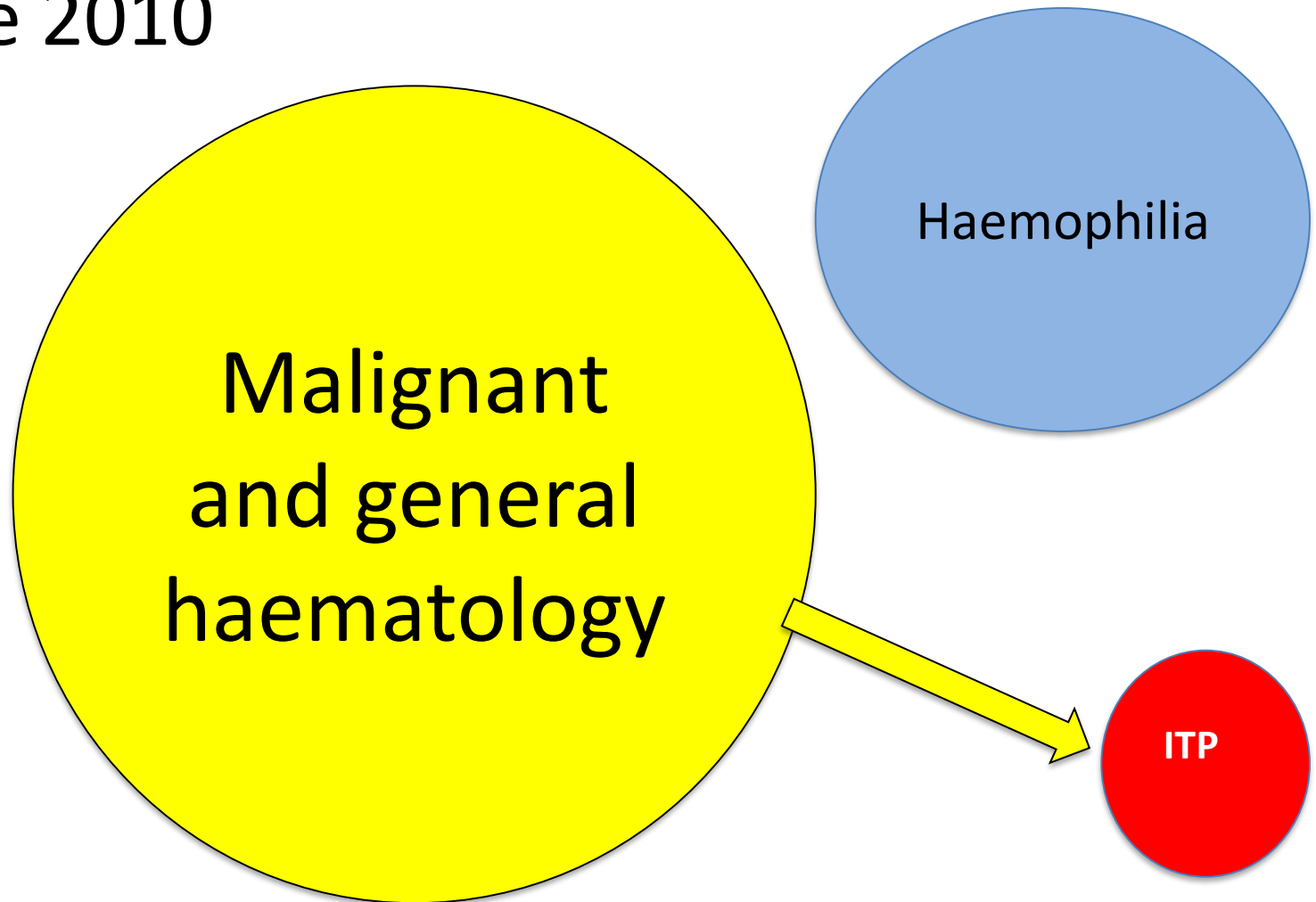
MRI Study: Can subclinical brain micro haemorrhages be detected in patients with thrombocytopenia using susceptibility sensitive MRI in order to better direct treatment. Inclusion criteria: At least one count $\leq 30 \times 10^9/L$ since diagnosis. Willing to travel to Hammersmith Hospital, London.

Paediatric Romiplostim Study: A Single Arm, Open-label, Long-term Efficacy and Safety Study of Romiplostim in Thrombocytopenic Pediatric Subjects With Immune Thrombocytopenia (ITP). This study is ongoing, but not recruiting participants.

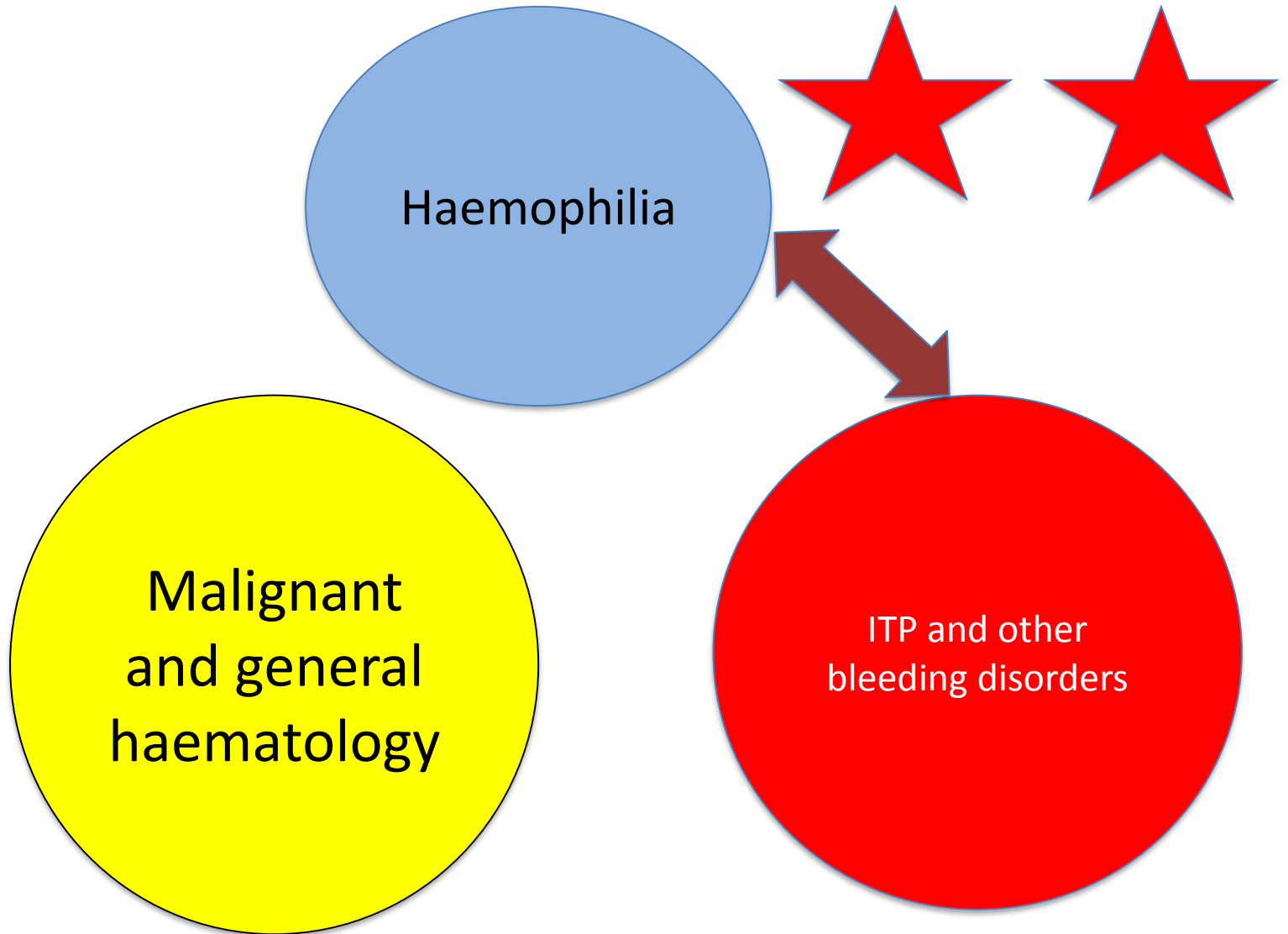
Very good networking between experts



Before 2010



Now



Haemophilia

Malignant
and general
haematology

ITP and other
bleeding disorders