MPDlife

The newsletter for people with MPDs
March 2013

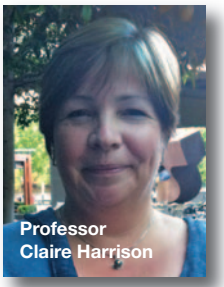
NICE recommendations for Ruxolitinib – latest news

IN the November 2012 issue of MPDlife readers were updated on the results of the US studies that led to the approval of Ruxolitinib as a new and effective drug therapy for myelofibrosis (MF). This drug has also been approved in Europe and has been available in the UK via clinical trial, through a local cancer drug fund or via an individual treatment application.

Results on the effectiveness of the drug have all indicated a new hope for MPD where many MF patients suffering with enlarged spleen, itch and fatigue responded well to Ruxolitinib.

As with all new drugs that are developed for use in the UK, as well as the stringent criteria for trials and evaluation which can take several years, one of the other crucial factors determining the final launch and general availability of any new medicine is the approval by the National Institute for Health and Clinical Excellence (NICE).

On 13th February 2013 NICE released draft guidance of their preliminary decisions not to recommend the use of Ruxolitinib. The independent Committee considered many factors in reaching this decision but concluded that whilst Ruxolitinib was clinically effective it could not be considered a cost-effective use of NHS resources compared with best available therapy for treating disease-related splenomegaly or symptoms in adults with MF.


Professor Claire Harrison

Professor Claire Harrison commented in response, 'The lives of patients affected by MF are improved with Ruxolitinib therapy. In many cases this improvement is dramatic with long lasting tangible benefits. There is now increasing evidence that Ruxolitinib therapy also prolongs survival in this difficult disease where we have previously had very limited options. This decision is disappointing for patients, their families and clinicians. However the work of NICE is crucial and we will work with NICE as well as patients and clinicians to ensure that as drugs are developed that appropriate information is gathered so that important new groundbreaking treatments like Ruxolitinib are made available where appropriate to patients in the UK.'

MPD Voice has also written to NICE, urging them to reconsider their decision as we know that this is a very important issue for all affected by MPD, especially as the latest results of the trials currently being undertaken are very positive.

Commenting on behalf of NICE, Professor Carole Longson, Health Technology Evaluation Centre Director said, 'It is disappointing not to be able to recommend this new treatment in our preliminary recommendations, but in order to do this we have to be sure that the treatment is both clinically and cost effective. The draft guidance is now out for consultation and I would urge all those with an interest in MF to comment via the NICE website.'

At the time of writing NICE had not yet issued final guidance to the NHS and was inviting comments which it would consider at its meeting in March, after which final guidance is expected to be published in June 2013.

NICE

- NICE was set up in 1999 to reduce variation in the availability and quality of NHS treatments and care – the so called 'postcode lottery'
- They use evidence-based guidance and other products to resolve uncertainty about which medicines, treatments, procedures and devices represent the best quality care and which offer the best value for money for the NHS
- The NHS is legally obliged to fund and resource medicines and treatments recommended by NICE's technology appraisals
- Every piece of NICE guidance and every NICE quality standard is developed by an independent committee of experts including clinicians, patients, carers and health economists

Update on UKOSS research into MPD and pregnancy outcomes

Dr Sue Robinson, Haematologist at Guy's and St Thomas' NHS Foundation Trust has played a leading role in the inclusion of the monitoring of pregnancy outcomes for MPD patients. She explained the background and progress of the study.

Launched in 2005, the UK Obstetric Surveillance System (UKOSS) is designed to be used to survey a range of rare conditions in pregnancy.

Pregnancy outcomes for women with rare conditions are difficult to study resulting in limited information about the natural history, prognosis, risk factors and evidence-based practice. UKOSS draws together clinicians from all consultant-led maternity units in the UK in a routine reporting system, allowing a straightforward way to study lots of conditions. The information gained from these may be used to inform counselling of women, development of guidelines for prevention or treatment and for service planning.

Anonymous descriptive data is collected through a monthly scheme and each hospital reports on conditions with an estimated incidence of fewer than one in 2000 births.

In 2010, with a grant from the Guy's and St Thomas' Charity, MPD was added to the survey, as historical literature suggested some association with increased maternal and fetal morbidity and mortality. Prior to that there had been no prospective national studies to estimate the incidence or outcomes of MPD in pregnancy.

To date 39 cases have been identified. The data collected will be analysed and presented in a paper. Results may provide prospective data upon which to base future management strategies. Dr Robinson hopes to also consider the potential to develop an ongoing UK based data collection system.

MPD voice

GUY'S & ST THOMAS' CHARITY

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Miracles do happen

patient story

Jennie Barnes, 23 has lived with essential thrombocythaemia (ET) since she was diagnosed at the age of 13. Last year she was told that this had transformed into leukaemia. In the weeks that followed Jennie wrote an honest and moving blog about the ups and downs accompanying that diagnosis and has kindly agreed that MPDlife can publish extracts.

January 29th

I should be in Paris right now. Instead I'm sat on my sofa getting ready to begin my journey to fight the dreaded leukaemia. Let me take you back 10 years and three months…

At the tender age of twelve I was your typical scrawny teenager, too skinny for my age and size, ginger hair and braces and as far from cool as you could get. The month before I had a couple of teeth taken out to fit my brace, I bled and bled, prompting my dentist to recommend a blood test.

The next month just before my thirteenth birthday there was a knock on the front door. Mum answered and it was the doctor. The doctor showing up on your front door, weird right! He asked if he could come in and advised us to take a seat. The next 5 minutes are a blur, but it involved my mum and dad crying, me crying and the doctor having that look on his face when they have to tell you bad news. They suspected I had a type of childhood cancer.

I was rushed to a children's hospital and placed on a ward of very sick children, all had lost their hair! I was prodded and poked and had lots of blood samples taken. I remember being taken down to have a bone marrow trephine/aspiration. I woke up and felt as though I'd been kicked numerous times and it hurt to stand, sit down or move at all. It turned out I didn't have leukaemia I had essential thrombocythaemia (ET).

January 30th

For the last 8 years or so I have carried on my life as any normal teenager would. I've worked hard, played hard, fallen in love, thrown tantrums, fallen out with friends, made friends, went to university and had numerous jobs. All this time, as far as I was concerned, I was healthy and normal! My symptoms ranged from hair loss, burning sensation in hands and arms, fatigue and general tiredness, shortness of breath, pins and needles and severe anxiety!

Until September last year I thought everyone felt these sorts of things. Turns out I was wrong. In September I discovered MPD Voice and the online community. They helped me realise that it's ok to say you're too tired and to ask for help and question the doctor.

Happy families – Jennie (right) with her Mum, Dad and sister

It's because of MPD Voice, that when my doctor asked, 'So how are you feeling today?' Rather than my usual answer, 'Fine'… I said 'Actually I don't feel fine, I'm tired, my bones ache and I feel rubbish.'

My doctor was great, consulting with a specialist in London about a new drug that might not make me feel as rubbish. But first I needed another bone marrow test just to be on the safe side! This was where it all started to go wrong for me.

The test results came back and that's how leukaemia became part of my life…

February 16th

Yesterday, Friday 15th February was D day, or so I thought. It turns out yesterday was the best day of my life. …I'm still struggling to comprehend exactly what happened. I may have to go back to when this all started.

In November when my results came back from my bone marrow biopsy I had 7% blast cells. Anything above 7% is leukaemia and requires immediate treatment. Therefore plans were started to perform a bone marrow transplant.

In a matter of weeks a donor/match was found for me from the Anthony Nolan bone marrow register. I started IVF treatment to freeze some eggs and made the decision to undergo my chemo and transplant at a different medical centre to where I had previously been treated. My new consultant wanted to do another bone marrow biopsy to determine how much chemotherapy was needed before we started.

On D day (Feb 15th), we got to the hospital and waited until the doctor called us in. This time my community liaison nurse was in the room along with my transplant co-ordinator. I assumed they were there for extra support and to talk me through the treatment plan.

The first question the doctor asked me was, 'So do you have anything to tell me?' I replied 'No'. He then said, 'Well neither do I'. My initial reaction was anger! What did he mean? I wanted details, I wanted to know when my treatment was starting. Then he said, 'I have nothing to tell you because you don't need a transplant, you don't have leukaemia!'

SILENCE. I didn't respond, neither did mum, dad or my fiancé John. What did he mean I didn't have leukaemia? How could I not? My donor was on stand by, my IVF had been completed, I'd had ECGs, scans, hundreds of blood tests. 'Jennie you don't have leukaemia!'

I cried; I sat there and cried. For the first time in 3 months I was crying tears of joy not sorrow. I still didn't believe him, so he showed me the test results, it was there in black and white. 2% blast cells. Normal, no leukaemia cells, no more than the average Joe has!

February 21st

In her most recent blog Jennie writes about the current challenges of taking medication that makes her feel ill and continues:

So after the happiest weekend of my life, you would think or expect it to be business as usual. Well it is apart from a few little niggles. The reality is that I now feel worse than before, the reason being I've started on a new medication or rather an old one for me, hydroxycarbamide.

For 10 years I have lived with this rare blood cancer and only now am I understanding that more needs to be done to make people realise that we shouldn't just brush it off as a 'blood disorder'. It is cancer and we shouldn't be afraid to talk about it and we shouldn't hide the fact that we feel awful on days when we do. We should make a fuss!

That way we can raise more awareness which in turn will raise more money for research to find better ways of treating the cancer as it isn't curable only treatable. People with MPDs have them for life, there is no remission for us.

Jennie is passionate about spreading the word about MPDs and concludes,
'The more people know about it, the more likely it is to fund research into better treatments'.

Jennie's work colleagues are taking part in the Wilmslow half marathon – www.justgiving.com/energizeJB

To read Jennie's full blog, including her IVF experiences, visit www.howdoyouplaythegame.wordpress.com

Benefits for people living with MPD

For some patients diagnosed with MPD, in addition to managing the physical and medical aspects of the condition, quality of life, money and ability to work are also serious concerns for patients and their families.

Below are a few considerations/benefits that may be applicable to families or individuals affected by MPD.

Working age benefits

- **Statutory Sick Pay** Can be claimed if unwell for at least four days in a row and paid by the employer for up to 28 weeks of sickness. Min average weekly earnings apply.
- **Employment and support allowance** Available for up to 12 months. Provides two types of financial help to people unable to work because of illness or disability. People may get either contributory-based or income related (means tested) allowances or both.
- **Personal Independence Payment (PIP)** From April 2013 this will replace the Disability Living Allowance. It will include a daily living component and a mobility component. For people currently receiving Disability Living Allowance reassessments are planned to determine if eligible for PIP.
- **Income Support** A means tested benefit available to people aged 16 to pension age, who are on a low income and have savings of less than £16,000.

Pension age benefits

- **Attendance allowance** Two levels of allowance for people over 65 who have difficulty looking after themselves. It is based on the amount of care you need, rather than any care already being received.
- **Pensions and pension credits** In recent years there have been many changes and factors influencing ages/rates and levels of state pension payments. For the latest information visit direct.gov.uk/pensions
- **Carers allowance** For anyone aged 16+ and who spends at least 35 hours caring for someone who receives Disability Living Allowance or PIP.
Visit www.direct.gov.uk/en/caringforsomeone for more information about this entitlement.

Please note that this is not a comprehensive list and is based on extracts from 'A Quick Guide to Benefits and Financial Help' produced by Macmillan Cancer Support. For the most up-to date benefits information consult with a welfare rights adviser.

Thanks to Macmillan Support for allowing inclusion of extracts of their literature. Further/fuller information can be found at www.macmillan.org.uk or they offer a free Macmillan Support Line and dedicated welfare rights team telephone: 0808 808 0000 Mon-Fri 9.00am–8.00pm

Epidemiology study gears up

MPD Voice has previously reported on plans to fund and launch an epidemiology study which may help in identifying causes of MPD

Now onto the next phase, the Chief and Principal investigators met in Belfast to discuss and finalise details of the MOSAICC Study. The study questionnaire has been designed using state of the art software and biological sample collection kits are ready and the pilot study will start recruitment in March 2013.

Eligible patients will be invited to take part by their clinician who will provide them with an invitation letter and information booklet at their next appointment. The collaborators are keen to encourage as many patients as possible to participate in the study as the results will be used to design the UK-wide study planned to start in 2014/15.

PhD student Glen Titmarsh has attended three conferences to present posters including a review of the incidence and prevalence of MPDs and the role of infection-related conditions and MPDs. Glen is preparing to submit these studies for publication.

The MOSAICC study team are also seeking to recruit 100 non-blood relatives or friends of patients with MPDs in Belfast and Southampton as a comparison group. Further details can be found at http://mosaicc.qub.ac.uk

Prof Mary-Frances McMullin, Dr Andrew Duncombe and Dr Lesley Anderson in Belfast.

ASH 2012

Professor Claire Harrison reports on the highlights from the December 2012 meeting.

ASH – American Society of Haematology, is haematology's most prestigious meeting attended by over 25,000 delegates. MPD continued to enjoy a high profile at the event.

Professor Harrison shares that, 'It's a time for hearing the latest data, networking with colleagues, and for planning future trials and collaborations.'

Target Haematocrit (HCT) in Polycythaemia Vera (PV)

The target HCT in PV has long been debated. Work suggesting a target of 0.45 was done at St Thomas' in 1978. At ASH 2012, Professor Barbui confirmed this target to be correct in the CYTO PV study. However it was noted that the white (or leucocyte) count was also lower in those patients with lower HCT and so could also be important.

JAK inhibitors

Updated results from the COMFORT 1 and 2 studies were presented, broadening data for durability of response, safety and adding exciting news for overall patient survival. Other JAK inhibitors may offer complimentary or additional benefits to Ruxolitinib and strong data was presented for two other inhibitors SAR302503 and CYT387.

Talpaz and colleagues evaluating SAR403503 in patients randomized in a phase II study and taking varying dosage per day indicate potential important results. This has been reported to be associated with allele burden and bone marrow fibrosis grade reductions.

CYT387 a small molecule inhibitor of JAK 1 and JAK 2, tested in a phase I/II multicentre study demonstrated improvements in splenomegaly and symptoms as well as a reduced need for transfusion.

Conclusions for JAK inhibitors

The current data suggests that JAK inhibitors impact MF beyond symptoms and spleen size reduction and offer major benefits to patients.

Upcoming results are also expected for the 3 year data from the COMFORT studies, the phase 3 study with SAR302503 as well as RESUME a controlled study with pomalidomide in MF and JAKARTA-2 a study of SAR302503 in patients who have been refractory or intolerant to Ruxolitinib.

If you want to read more there is a webpage for the meeting itself and MPN forum also published an ASH report.

See Glossary on back page.

First ever summer concert

4th July, Savile Club, London W1

A host of gifted young musicians will play in support of MPD Voice, giving their time and talent for free. The evening comprises drinks, canapés and a short address by Professor Claire Harrison, Chair of MPD Voice followed by a concert in the magnificent 'Rococo' Ballroom. There will be a short talk given by Will Self (MPD patient), the English author, journalist and television personality and a fundraising auction and raffle will take place.

Profits will go directly to our epidemiology study, so please support MPD Voice by buying tickets to the concert or, if you have anything to donate to the auction or raffle we would love to hear from you: tickets to an event, vouchers to a restaurant, a designer piece of jewellery or accessory, a personalised tour to a place of interest, membership to a club that means you can donate a day of sport, please let us know as it can all be used to support MPD Voice.

For tickets or donations for auction contact Rachel at fundraising@mpdvoice.org.uk

Glossary – ASH 2012

JAK1 and 2 2 members of a family of Janus Activated Kinases. These bind to receptors on the surface of cells and transmit messages. JAK1 and 2 are important for sending messages to make more blood cells.

COMFORT trials These were the first ever large scale so called phase III studies (testing new drug against either sugar pills or standard care) in MF. The studies involved ruxolitinib or INC424 a JAK1 and 2 inhibitor and their success led to the approval of ruxolitinib for treatment of MF.

Allele burden Refers to a measurement of how much of a gene is abnormal. For JAK2 this would be expressed as the % of JAK2 which is JAK2V617F.

Splenomegaly Enlargement of the spleen. A common finding in MPD and a particular problem in patients with MF where spleens can be more than 20x normal size.

Fantastic fundraising

Rachel Bridgman, our fundraising co-ordinator, is excited about the amazing activities planned by patients and supporters for the forthcoming year. She says, 'We have pledges of all sorts of wild and wonderful things, most importantly of course people have pledged time, energy and commitment to supporting MPD Voice.'

David Blenkinsopp is planning a Golf Day at Eaglescliff Golf Club, Stockton-on-Tees. Contact fundraising@mpdvoice.org if you are interested in taking part.

Kathryn Mcgregor and 3 university flatmates are taking part in the Yorkshire 3-peaks: www.justgiving.com/Kathryn.Mcgregor

Suzie Whaites is taking part in Lincoln's half marathon: www.justgiving.com/suzy-whaites1

Louise Simpson is parachuting for MPD Voice: www.justgiving.com/louisejaynesimpson

Andrea Headech is shaving off her hair if she is pledged enough to do it! Sponsorship details to follow on the website – good luck Andrea ☺

Energize Recruitment Solutions members of staff will be running the Wilmslow half marathon in support of work collleague Jennie Barnes (see patient story): www.justgiving.com/energizeJB

Visit the website for an update of fundraising events throughout 2013 www.mpdvoice.org.uk

If you would like to support MPD Voice but don't have time or energy to take part in an event yourself why not sponsor one of the above? If, however, you would like to take part in an event, or you are planning your own event this year, please email Rachel at fundraising@mpdvoice.org.uk She can support you by sending fundraising information, collecting pots, sponsorship forms, t-shirts etc.

Forums 2013

- **May 2013** London
- **16 November 2013** Patient Day, London

If you would like us to run a forum in your region contact info@mpdvoice.org.uk

For upcoming and additional 2013 dates please keep an eye on the website www.mpdvoice.org.uk

Thank-you

Our continued thanks to the **Samuel Sebba CharitableTrust** whose support has enabled us to maintain the increased distribution of our newsletter and number of regional forums.

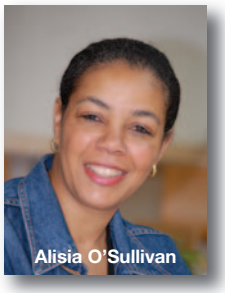
Thanks also for recent donations from **Aston Marton** who donated £1,667 to the epidemiology study and **Alan Firmin Ltd** who donated £630 on behalf of one of their colleagues who has MF.

Visit our **News and Events blog**: www.mpdvoice.org.uk/news-events or visit our Facebook and Twitter pages

MPDlife

- Want to be featured in our patient story?
- Do you have tips to share with readers on managing MPDs?

If so, please email the editor at the address below.



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