

mpd-support



GUY'S &
ST THOMAS' CHARITY
Charity No. 251983

The newsletter for myeloproliferative patients

April 2008

JAK2 inhibitor trials open in the US and the UK

Are the inhibitors working? Dr Claire Harrison and Ann Marie Jahn report from London and New York

Carol is a woman in her mid-fifties with myelofibrosis (MF). She and her husband Jack (not their real names) attended the Myeloproliferative Disorders (MPD) Patients' Symposium in New York last November.

At the pre-meeting breakfast, Jack explained why they had come to the symposium: "My wife has MF and the doctor told her to get her affairs in order. We're just hop-



The JAK2 molecule

ing to hear some good news today." Jack patted his wife's hand as she looked down into her coffee cup.

The New York symposium covered a wide range of topics, but the most intense discussion focused on treatment options for MF, with data from the inhibitor trials just emerging. The haematologists speaking in New York debated the merits of

various options, at times arguing in heated tones.

Just what are the options for patients with MF? Bone marrow transplants (BMTs) involve tremendous risks but are the only known way to cure an MPD; they remain the treatment of choice for certain patients today.

The newer, unproven alternative is therapy with JAK2 inhibitors. Dr Tefferi of the Mayo Clinic argued in favour of these drugs. "If you were my brother or sister, I'd tell you to forget the BMT. My patients taking the inhibitors say they've never felt better in 30 years," he told the audience.

Although these comments are clearly hopeful, it's by no means certain how well these early inhibitors will work. Two compounds have been tried in patients with MF to date. The first of these, called CEP70, showed little or no activity in treating MF. A second inhibitor called Incyte (INCB108424) is under trial at the MD Anderson Cancer Center in the US and has shown more encouraging results. In November Dr Vestorvek of MD Anderson reported a striking re-

sponse in patients taking Incyte. The compound reduces enlarged spleens, but so far does not show diminished marrow fibrosis.

Seven additional compounds are poised to enter clinical trials to treat MF. Many more drugs are likely to be available in the near future, giving all of us reason to be optimistic that MPD treatment options will enter the 21st century, bringing hope to people like Carol and Jack.

JAK2 trials in the UK

When do trials begin? Trials are beginning now

Where will trials take place? In Belfast, Cambridge, London, Oxford and Sheffield

Who's eligible? People with intermediate or high-risk MF

How is the medication given? As a drip

What if I want to enrol? Please speak with your haematologist

An empowered patient pursues all possible options

It isn't always simple deciding on a course of action when you have an MPD. Ann Marie Jahn explains how one man is considering his choices as he continues to help others in need

This month the JAK2 inhibitor trials opened in the UK – thrilling news for anyone who is JAK2 positive and for people with myelofibrosis (MF). In this hopeful atmosphere people with MF face what can be difficult, even agonizing decisions.

Deciding on treatment options can be a fraught process, as patients and families weigh physical risks and even financial implications.

Robert Key discovered he had myelofibrosis four years ago. Like many other people with an MPD, his first symptom was a problem with his feet. His GP referred him to a podiatrist, who was mystified and unable to make a diagnosis.

Robert continued to request help from his GP, who remained perplexed until Robert reported that he also felt fatigued and was

experiencing a decrease in libido. This information provided the clue that solved the mystery. Severe fatigue might indicate anaemia; foot problems might indicate impeded blood flow. It was becoming clear that Robert had a bone marrow disorder.

'I try to conduct my life as I always have, but I pare back to the essentials'

His GP referred him to a hospital in London, but he wanted more specialised advice. He travelled to New York to seek help from Sloan Kettering Cancer Center. In their view Robert was not yet ready for a bone marrow transplant (BMT). He was eventually referred to St Thomas' Hospital where he was treated with a course of thalidomide, prednisone and erythro-

poietin.

Robert is someone with special expertise in fighting for patients' rights; he believes in actively pursuing the treatment you prefer. He is Executive Director of the Elton John AIDS Foundation (EJAF), a charitable organisation

which assists people infected, affected and at risk of HIV/AIDS. The foundation's mission is to empower all people in these situations – to alleviate the hardships they face, to improve their quality of life, to enable them to live with dignity and to exercise self-determination. Robert was recently recognised with an MBE from the Queen for his work in achieving these goals at the EJAF.

One of the foundation's guiding principles is that everyone has the right to access the best medicine. Robert knew from experience that he would have to seek out experts to get the best care for his myeloproliferative disorder. He decided he would "not take this death sentence

lying down."

As his illness began to progress, he reviewed the options with his haematologist: would it be best to try the inhibitors or to opt for a BMT? Robert felt that a discussion with specialists in the US could clarify his choices.

His haematologist at St Thomas' arranged a four-way conference call with Robert and St Thomas' in the UK and Drs Ronald Hoffman and Ruben Mesa in the US. Dr Hoffman is a renowned BMT specialist at Mt Sinai Hospital in New York; Dr Mesa is leading research on JAK2 inhibitors at the Mayo Clinic in Minnesota. The specialists debated which treatment offered the best hope for survival.

Robert explains why he sought additional opinions and options from a US hospital: "In America patients are screaming for treatment and pursuing access to a new range of drugs that will keep them alive. They don't sit back and wait." However, as a UK citizen, Robert did not have the private medical insurance that would cover the cost of a BMT in the US. He faced an



Robert Key receives his MBE from the Queen

excruciating decision: whether to sell his home in London and liquidate his assets to fund phenomenally expensive private treatment in the US. The hospital would require him to move to the US for at least six months and to pay all his medical expenses out-of-pocket. The cost might top £200K for the treatment alone, excluding housing and travel expenses.

When we spoke, Robert was weighing his options. “It’s very difficult determining what long-term plans I should make, for instance what to do about my house. Would it be best to move to something smaller? Should I move now while I still have the energy? Should I sell my home and spend what I’ve got on care in the US?” Robert asks.

Although it’s an optimistic time for people with MF, with more choices becoming available, people must

still cope with a gradual stripping-down of their possibilities and their lives.

In Robert’s role at the EJAF, he travelled to a number of countries in the developing world, educating local people and helping them obtain medical care. This was work he found tremendously fulfilling. One of the great losses he has experienced since his diagnosis with myelofibrosis has been curtailing his work in the developing world. He found he was too unwell to cope with the demands and risks of frequent travel to these countries.

Robert’s time abroad has given him perspective on how a person’s attitude can change the course of a disease. “Traditional healers – people we once called ‘witch doctors’ – have a tremendous impact on people. The healer’s message is so strong that you may live or die based on what he says. If a traditional

MPD research in the UK

- **What is the UK’s role in JAK2 research?** The UK has a strong collaborative group leading the way in worldwide MPD research. Prof Tony Green of the Cambridge Institute for Medical Research was one of the discoverers of the JAK2 mutation. Now the largest-ever ET trials are being run from the UK by Dr Claire Harrison and Prof Tony Green
- **Will the best drugs be available in the UK?** Yes. Drugs under trial in the US which show potential benefits in treating MF will likely come to Europe for trial. There will also be drugs tested first in the UK

healer tells a patient that he will die, that patient often goes home and dies within a few days. The patient’s belief about what will happen is powerful enough to alter the course of the illness. You must therefore maintain a positive attitude.”

“I’ve decided to keep in work. I try to conduct my life as I always have, but I pare back to the essentials. Plans and dreams must now go on

hold. My partner and I have talked about the future, about the possibility of my death, about my will. You do have the idea that you are tempting fate if you discuss your will. But you have to talk about what’s going to happen.

Gay people changed the face of medicine in the UK in the 1980s by fighting to obtain adequate care during the early years of the HIV/AIDS crisis. That spirit – fighting to get the best care and stay alive – applies to MPD patients as well. “Have you made a commitment to die? If not you must fight to keep living. Never take anything as an absolute, closed scenario,” says Robert. ■

As we went to press, Robert enrolled in the JAK2 inhibitor trials at the Mayo Clinic (US). We wish him every success with his treatment.

Treatment decisions – factors to consider

- Learn more about your illness. Read up, do your research, and make sure you’re aware of all the options
- Consider side effects and success rates when making treatment decisions
- Your age and your general health may affect your preferences. You may prefer a more or less aggressive course of therapy
- If you live with a partner, will your choice of treatment affect him or her?
- Are there any financial considerations?

Expert Briefing

Dr Ruben Mesa advises how to fight your illness with nutrition

QI've read that pomegranates contain powerful antioxidants. I try to eat a pomegranate every day. Can this help me feel better? I'm 34 years old and I have ET.

AWe've all heard a tremendous amount in the press about the benefits of eating particular 'magic' foods. This is an intriguing notion, but so far scientists have not identified any particular supplements that can 'treat' or 'cure' an illness. The best approach to nutrition is to eat a balanced diet. Select plenty of fresh fruits and vegetables, and be particularly careful to eat enough calories and protein if you are unwell. This balanced approach will help your body fight illness. Rather than focusing on one product, you should choose a broad variety of protective foods. Don't forget that it's important to stay hydrated. Many of us enjoy tea or coffee or an occasional glass of wine. It is fine to enjoy these drinks in moderation, but take care to drink enough water as well. All the best, Ruben Mesa MD ■

You can find more information on treatments at www.mpd-support.co.uk

Please note that nothing contained in this newsletter is intended to constitute professional advice for medical diagnosis or treatment. You should always seek the advice of your physician or other qualified health provider prior to starting any new treatment or consult them on any questions you may have regarding a medical condition.

Cord blood pros and cons

BY ANNE FRANCES FERMOR

When my baby was born four years ago, a thought flashed into my head: could my baby's stem cells cure my MPD? Events moved fast that day, and I was unable to arrange for his cord cells to be collected. A once-in-a-lifetime opportunity disappeared with my son's premature delivery.

Now there is more than one way to collect and store stem cells for future use. Companies in several countries can store stem cells from baby teeth, from a woman's menstrual blood, from umbilical cords and from embryos left over from in-vitro fertilization. Parents in the UK can donate their baby's stem cells to an anonymous blood bank, or they can store the cells privately for a fee.

Reading the advertising on cord bank websites, it seems these cells can cure anything. The New England Cord Blood Bank in the US claims on their website that all MPDs can be treated with stem cell transplantation. They seem to imply that stored cord blood cells will treat or cure these disorders.

But a recent *New York Times* article warns it's not clear whether the cells will be useful or not. The *Times* quotes Christopher Scott, director of the Program on Stem Cells in Society at Stanford:

“In the stem cell area, we have a problem with truth in advertising. Some of these [stem cell storage] companies are skirting right on the edge of what's truthful and what's vaporware.’ The companies, some of them small and financially shaky, are capitalizing on the excitement surrounding stem cells.”



It could take many years for certain stem-cell treatments to become available. It's also worth bearing in mind that stem cells from one child may not be a complete genetic match to either parent or any of their other children. Leftover in-vitro embryos may not genetically match either parents or children.

What's more, extracting and storing stem cells is expensive. Cells4Life in the UK charges £1495 to collect, process and test stem cells. The fee includes the first 25 years of storage.

If you do choose to store cord blood cells when your baby is born, you'll want to plan ahead. Some services will send you a kit by post. Your doctor or midwife collects the cord blood for you when your baby is born. The service then picks up the blood and sends it to the cord bank by courier.

“My tooth feels wiggly,” said my now four-year old son this week, pointing to one of his baby teeth. I think to myself, “Just what sort of magic dust might be in that tooth?” ■

Communicating with family and friends

Talking about your MPD can be difficult, but others understand better if you know how to present your situation. Helen W Wong gives tips on connecting with the people around us

It's happened to all of us: We explain our MPD to family and friends and get a blank, confused look in response. Many people in our lives are unfamiliar with MPDs. Try these techniques for easier communication:

Stay calm You communicate best when you speak with a calm, even voice. Try to avoid sounding defensive if at all possible.

Explain concepts Be prepared to explain concepts that have be-

'Be prepared to explain concepts that have become familiar to you'

come familiar to you. Your listener may not know what a platelet is or how blood cells are made in our bodies. Use simple language and

avoid medical jargon.

Offer a comparison Compare your symptoms to things your listener can readily understand: the fatigue that comes with the flu, headaches from migraines or itchy skin from chicken pox.

Talk about your ups and downs Others usually see you when you're feeling your best (when you go out or go to work), not when you need to stay home to rest. Let others know that your symptoms can vary significantly from day to day.

Set limits Clarify that you may need to take time for hospital appointments, to rest and to deal with symptoms. Let your friends and family know that you value their understanding and support.

Build in flexibility Talk with friends and family about your need to stay flexible. Alert them that you



Helen W Wong recently completed her MSc in Health Psychology

may need to cancel plans at the last minute if you're feeling unwell.

Let them ask questions Some people in your life may want to understand more. Let them know that it's OK to ask questions.

MPDs are rare diseases, so it's not surprising if others feel confused. Good communication will give them greater insight and empathy for your experiences. ■

Healthcare websites offer practical tips on living well

It can be hard to find medical websites you can trust. Here are three reliable resources to try:

If you'd like advice on eating right and getting strong, you can't do better than the world-renowned Mayo Clinic (www.mayoclinic.com) which offers clear information on medical conditions plus tips for staying well. Click on "Healthy

Living" for tasty, nutritious recipes from the Mayo Clinic's cookbook. You can even view fitness videos: you won't need a personal trainer.

If you are looking for a fresh angle on daily living, Caring4Cancer (www.caring4cancer.com) provides practical ideas such as writing a journal, tracking your symptoms, talking with children

and grandchildren and creating a healing environment for yourself.

If you need help with communication, Cancerbackup (www.cancerbackup.co.uk) gives detailed advice on topics like talking with family about illness or surviving as a caregiver. This is also the place to look for a frank discussion on fatigue and intimacy. ■

Fight fatigue with exercise – seven steps

Dr Ruben Mesa of the Mayo Clinic explains how activity can boost your energy level

Dr Ruben Mesa treats and studies MPDs as a haematologist at the Mayo Clinic (US). In his years working with MPD patients, he's found that fatigue is the most common problem patients face.

"Physicians don't always appreciate just how fatigued MPD patients can feel. They often overlook or undervalue the symptoms that MPD patients report," he explains.

In the future, better treatments may hold the key to overcoming symptoms, but exercise is a great way to feel better today. Dr Mesa suggests the following approach:

1. Define your goals Writing down your objectives will build your motivation. Your goals can include improving muscle tone, sleeping better and feeling less fatigue. It's useful to define a specific goal that you can measure, such as walking to the bottom of the garden every day or running a 10km race.

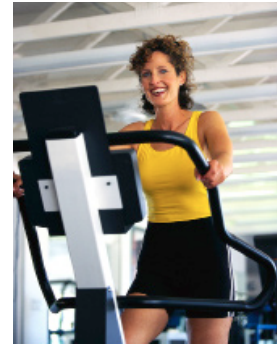
2. Ramp up slowly "The classic error in any exercise programme is doing too much too soon – it's the 'New Year's resolution phenomenon,'" says Dr Mesa. You will need to increase activity

very slowly over time to avoid exhausting or injuring yourself.

3. Team up with your GP "People with MPDs cover a broad range, so it's important to create a bespoke programme for your particular situation," explains Dr Mesa. You should develop a reasonable and modest plan with your GP, taking into account any limiting factors such as a history of clots. MPD medications are not a major hurdle to physical activity, but do check with your physician before you begin.

4. Gather your tools "It can be a challenge to implement a programme at home," says Dr Mesa. New tools can help you get started. Use a pedometer to record the number of steps you walk each day. Keep track of your progress in a logbook. Invest in a comfortable pair of walking shoes and comfy, loose-fitting exercise clothing.

5. Measure gains over time You may need to wait many weeks or even months to see an improvement in your energy level and physical condition. That's the case for healthy people and it's equally true for those with MPDs. Increase



your level slowly and gradually, then measure your progress against your goals every few months.

6. Push the limit "The net change over your baseline has the biggest impact. Over time you can push the limit further and further out," says Dr Mesa. Check back with your GP and haematologist and redefine your goals when you feel ready to take on some new challenges.

7. Enjoy your new energy It's clear from the latest research that even a very modest exercise programme can yield tremendous results – you can reduce fatigue, become more mobile, improve circulation and get more oxygen to your tissues. You may never feel quite as good as you did before you had an MPD, but you can feel a whole lot better. ■

Fatigue the most common problem for MPD patients

Dr Mesa of the Mayo Clinic in Rochester, Minnesota (US) recently completed an online quality-of-life survey of 1200 MPD patients from 30 countries. The survey looked at patients' diagnosis, blood counts, age and other factors, and asked patients to rate how tired they felt on a "fatigue inventory."

Nearly 90% of respondents reported feeling significantly more fatigue than healthy people. Dr Mesa also found that MPDs are likely to cause a range of symptoms that are not easily explained by a patient's blood counts – a sign that we may not know everything there is to know about these mysterious disorders.

MPD Support sets ambitious goals

Mother-and-daughter team will drive fundraising efforts for the charity

People with illnesses such as breast cancer, multiple myeloma and MPDs often feel frustrated at the slow pace of research. Now people facing these disorders are taking action to control their destiny: patients' support groups are gaining power and becoming a force in medicine. These groups help everyone access the best care. What's more, they take matters into their own hands, raising money and offering grants to researchers.

Our organisation too has a critical role to play. We must ensure that all interested patients have equal access to the JAK2 inhibitor treatments and that UK research moves forward at a fast pace. We aim to raise £25K in 2008 to support these goals.

Two fundraising pros recently joined the MPD Support team: Hira Sisodia, CBE (who was diagnosed with ET in 2006) and her daughter Natasha Hyatt-Khan. Hira is the founder and president of the Royal Kingdom Foundation Trust, a humanitarian organisation dedicated to the well-being of children throughout the world. She and her daughter raised the funds to establish an



Hira Sisodia CBE (left) and her daughter Natasha Hyatt-Khan (right) will lead 2008 fundraising efforts

organise a dinner for the Motor Neuron Disease Association while still in the sixth form. She has since run numerous large-scale events and worked as a tele-fundraiser for several charities.

The pair are planning several large-scale events for MPD Support in 2008-09, including a Bollywood fashion show in August, a Hyde Park walk in October 2008 and a Valentine's Ball in February 2009.

"Everyone can get involved and help us to reach our goals, either by joining the fundraising committee or by organising individual events. Fundraising is highly rewarding and is a fantastic way to make a positive contribution whilst having a lot of fun," Hira explains.

There are many simple ways in which individuals can raise funds. Small events such as cake sales, car boot sales or sponsored walks can have great impact. Everyone can participate and keep MPD research moving forward. We look forward to seeing our mother-and-daughter team push MPD Support's fundraising efforts ahead in 2008. ■

If you'd like to organise a fundraiser yourself, MPD Support provides all the assistance you need, including sponsorship forms, promotional materials and more. Please contact us at info@mpd-support.co.uk for more information.

Runner gets fit; raises funds



Chris Sifleet triumphs at the 2007 Race for Life

My name is Chris and I'm 48 years old. This column is about my trials and tribulations as I try to get fit and raise funds for MPDs. I'm running in memory of my father, who died of myelofibrosis (MF) in 1991.

My dad was an ex-Marine – he and doctors rarely met. He came to discover he had myelofibrosis in a roundabout way. One day, he climbed up on a stool to change a light bulb in the lounge. He liked to work Marine-style, wobbling on the stool whilst there was a perfectly good set of steps in the cupboard. My Mum noticed he looked a bit yellow. "I've not been feeling well," he said.

We knew instantly that something was wrong. He was soon diagnosed with MF and began blood transfusions. His condition deteriorated in 1991 and he died of MF that year.

Now I've decided to do something useful in memory of my father. I ran the 2007 5km Race for Life and I'm now training for the 10km Great Scottish Run in September. More about my races and my dad in the next issue. ■

Would you like to sponsor Chris? Just drop us an email at info@mpd-support.co.uk

'As the JAK2 trials open we have a critical role to play'

orphanage in the Philippines and to provide housing and medicine for many homeless children in several developing countries.

Natasha attended Oxford University and is now a student at the London School of Economics. Her involvement with charity work began early in life when she helped or-

Blood tests close to home

BY JOANNETONKIN RDE

Do you need to make frequent trips to the hospital for blood tests? Patients often travel a long distance to come to clinic. It can mean taking time off work, taking your children with you, or needing help from a friend or partner.

It can be costly to travel – train fares, petrol, taxi fares, child care and parking fees add up quickly, and once patients arrive in clinic, there is often a long wait.

It's expensive, annoying and just plain tiring. Ever wished you could have your blood test closer to home?

Colchester General Hospital recently created the post of Nurse Consultant to improve this experience for patients. Patients with stable disease receive their blood forms by post, then have their

blood drawn at their GP's surgery, walk-in centre or local cottage hospital near to home. The Nurse Consultant phones the patient at a pre-determined time to discuss their results and current status, then sends their GP a letter following the consultation. It works very much like a normal outpatient appointment.

A patient satisfaction survey showed that patients who participated felt the system was effective and convenient – in fact 92% of patients said the telephone consultation worked very well. This new system has two benefits: stable patients receive appropriate and timely care close to home, and the hospital has more capacity for patients who need in-clinic care – good news for all concerned. ■

New MPD patient leaflets coming in May '08

If you prefer to read information in print, from next month you can find printable leaflets on our website. These describe the three MPDs and treatment options in easy-to-read language and will be available in large print for-

mat. Our 2007 leaflet is available to download now. You'll also find a how-to guide for organising your own fundraising events and other new content. Please visit www.mpd-support.co.uk – these downloads are free to all. ■



"This is a second opinion. At first, I thought you had something else."

Our next issue

- First results from the JAK2 inhibitor trials in the UK
- Young women with MPDs face difficult pregnancies and sometimes miscarriage
- Nutrition: are there supplements that make all the difference?

MPD Support News

GUY'S & St THOMAS' CHARITY

Charity No. 251983

Editor: Ann Marie Jahn

Medical Adviser:

Dr Claire Harrison

Assistant Editor: Roy Farndon

Contributors:

AF Fermor, Dr Ruben Mesa, Chris Sifleet, Joanne Tonkin, Helen W Wong

Contact MPD Support care of:
Dept of Haematology
St Thomas' Hospital
Lambeth Palace Road
London SE1 7EH
Telephone: 020 7188 2742
Email: info@mpd-support.co.uk

We welcome your letters. Please write with your suggestions to our postal address or info@mpd-support.co.uk

You can also contact the Guy's and St Thomas' Charity at info@gsttcharity.org.uk or visit their website for more information: www.gsttcharity.org.uk

Please note that nothing contained in this newsletter is intended to constitute professional advice for medical diagnosis or treatment. You should always seek the advice of your physician or other qualified health provider prior to starting any new treatment or consult them on any questions you may have regarding a medical condition.