

## New drug showing promise in ET and PV

Ann Marie Jahn talks to UK study coordinator Jo McAllister about hopes for new treatment vorinostat

Fifteen years after learning he had polycythaemia vera (PV), a patient has found in the last few years that his red cell counts have increased, requiring increased doses of medication. But he can't tolerate these larger doses and is suffering side-effects. He's wondering what he can do now.

This story represents the experience of many people with myeloproliferative disorders (MPDs), who may over time develop more active illness or suffer long-term side-effects, leading them to feel increasingly unwell. Better treatment options could improve quality of life for these people. One drug showing promise is vorinostat, a drug produced by Merck

& Coand also known under the brand name Zolinza®.

Trials have shown that vorinostat decreases tumour cell growth and increases cancer cell death in certain cancers. In the US, vorinostat is used to treat white cell cancers; in the UK it is under trial in patients with myeloma. The drug has also been shown in the laboratory to reduce cell growth in the presence of the JAK2 mutation, which appears in many people with MPDs.

In 2009 MPD Support began funding a UK-based vorinostat study together with Merck & Co and other funders. The trial is offered in four locations in the UK: Belfast, London, Cardiff and Birmingham.

MPD Support's contribution covers the cost of research assistant Jo McAllister, who coordinates the vorinostat study in the UK.

Ms McAllister began her working life assisting a pharmacist and rose quickly through the ranks, becoming a clinical trials coordinator in 2006. She has coordinated several well-known studies, including the trial of the drug Gleevec® for leukaemia and a breast cancer treatment study.

Ms McAllister feels confident that the vorinostat trial will benefit participants. "Researchers want to learn whether vorinostat can



Trial coordinator Jo McAllister

help people who aren't responding well to other treatments. Vorinostat reduces a patient's platelet count – sometimes even too much – but the side-effects appear to be mild," she explains. Side-effects include indigestion, diarrhoea and more rarely blood clots. Patients in the study will be required to take extra blood tests but not bone marrow biopsies. They will be asked questions about their experiences and symptoms in a questionnaire.

Ms McAllister finds her work very worthwhile. "Many patients send thank you notes – they feel better and have an improved quality of life. It's fulfilling for me to know that we can offer better treatments to people who truly need them." ■

### Learn about trials

Want more information about trials? Read more here:

On MPD Support's website at [www.mpd-support.co.uk/research.htm](http://www.mpd-support.co.uk/research.htm)

On the US National Institutes of Health website at [www.cancer.gov/clinicaltrials/search](http://www.cancer.gov/clinicaltrials/search)

Read more about vorinostat at Merck & Co's website at [www.zolinza.com](http://www.zolinza.com)

## Young professional reflects on her diagnosis at age 12

Says Gina: "Consider how you might overcome barriers. Use them constructively to achieve your dreams."

My name is Gina and I have essential thrombocythaemia (ET). I am one of the rare people to be diagnosed with a myeloproliferative disorder (MPD) as a child – I was diagnosed at the age of twelve. Today I am 24 years old and I've completed undergraduate and graduate degrees in the disability sector. I lead a busy and active life working with disabled people.

I came to be diagnosed with ET through a coincidence – it was something of a twist of fate. I was born in Athens. Greek families tend to be very protective of

their children – and my mother had more worries than most. My sister was anaemic as a child and needed to have her blood tested quite often. When my mother took my sister to the hospital for her tests she always brought me along as well. I saw my sister's blood tests as a good way for me to escape from school for a few hours. I had no sense that there was anything wrong with me.

On one of these visits I had a blood test as well, and strangely the results came back showing that I had too many platelets. The microbiologist who checked my results suggested that

I be re-tested to make sure. Several blood tests followed, but my platelet count was always the same – over 1500.

I wasn't too worried. I was just twelve years old and I could not understand what all this was about. My parents spoke with my doctors, but they had difficulty obtaining an understandable explanation of why I had this disorder and what the implications might be.

My experiences during those early years with an MPD took place within a certain cultural and societal context. In Greece, family has a central role in society. Parents tend to protect their children, wrapping their offspring in cotton wool and providing every kind of support throughout their lives – physical, emotional and financial. My family is small, but we have strong, close relationships with one another.

After my high platelet count was discovered, for a time I continued to visit my local doctors, but at a certain point my doctors and my parents decided that the most suitable place to treat me was the haematology-oncology department of a children's hospital in

Athens. At the hospital, I sat in the waiting room together with children suffering from leukaemia. It was at that point that I began to feel worried that something serious was definitely wrong with me. When I missed days from school my parents would tell me, "It's because you have too many platelets."

Even if it's not such a long time ago, I find it hard to recall how it was living with ET as a child. I sometimes think that my poor memory of this time in my life is a mechanism that I have developed to protect myself, which may be why I have very selective memories from this period. I remember being a very active and sociable kid. I played basketball with my friends, cycled, swam, climbed up trees. I was just a normal, playful child. Then my doctors started setting limits in my life. I had to try not to hurt myself while playing. I was told to be more careful, not to run too much, to drink water constantly, eat carefully and to follow many strict rules.

I can remember the experience of a bone marrow biopsy – done without anaesthesia in a day unit because there



Gina now works with people with disabilities



were no rooms vacant. I vividly recall insisting on another occasion that I not be given full anaesthesia because I did not want to have to stay in the hospital while I recovered. Even now I feel depressed if I have to stay in a hospital overnight.

My parents, both well educated, began to do some research, and they concluded that the Greek health system could not provide the care that I required. My parents were unable to find any haematologists in Greece with an expertise in MPDs, so they decided to look for specialists abroad. When my parents told me that we might visit a hospital in England I was a bit scared but also quite happy to travel to another country for the first time. I remember how my parents acted when we first planned to visit doctors in London. They talked it up as a wonderful holiday, during which we would just have to spend a few hours seeing doctors.

I was treated with anagrelide at first, and I began treatment with interferon injections when I was sixteen, just I was preparing to sit the national exams to enter university. The Greek educational system is very competitive. I came home from school late

every night and then had to do my injection. Exactly five hours after I had my medicine the side-effects would kick in and wake me during the night – fever, shaking and severe pain in the legs. With side-effects and school pressure I found it difficult to cope, but I just had to handle it.

I can offer some advice to the parents of a child with an MPD: let your child know that being different is not bad. They must find the way to live with what they have. I've found it very helpful to share my feelings with family and friends and to have their support. Talking things through is important during those times when you feel that what is happening to you is unfair. I also believe that anyone with an MPD should just try to live as normal a life as possible. Your condition may throw barriers into your life – but you don't need to overestimate the impact of these obstacles. Consider instead how you might overcome these barriers and use them in a constructive way to accomplish your dreams.

I'm now an adult with an MPD and I lead as normal a life as possible. I don't feel my disorder affects my relationships with those



Gina (left): "I lead as normal a life as possible."

close to me, nor has it altered my plans and dreams. I want to travel around the world conducting research to help people with disabilities who live in institutions. My dream is to close down institutions so that everyone can be included in the community.

I am convinced that my own health condition has influenced my aspirations. Having an MPD has helped me keep my feet on the ground, even given me an asset. My experiences have led me to choose a career in working with people with disabilities. Living with ET as a child has given me a deep understanding of people facing disabilities. As

an adult I can handle my own feelings, and this strength enables me to help others. I am an empathetic person, but I don't feel pity for people who face severe difficulties. My work in the disability sector has led me to understand that there is a distinction between impairment, an attribute of the individual body or mind, and disability, a relationship between a person with impairment and society. I believe the same model can be used in thinking about long-term health conditions such as mine.

I won't allow my impairment to turn into disability. And I hope you won't allow yours to either. ■

# Cholesterol – why it matters in MPDs

Lianne Kolirin investigates why it's important to keep cholesterol in check, and how you can take control

Anyone with essential thrombocythaemia (ET) or polycythaemia vera (PV) faces risks, and the most common is blood clots. People with ET or PV are at risk because they have "sticky" blood – their blood is crowded because of too many cells. As a result they are much more likely to experience blood clots, heart attacks and strokes. People with myelofibrosis can have problems with cholesterol as well. Given these risks, it's essential to reduce any other factors contributing to blood clots.

One such factor is cholesterol, a waxy, fatty substance made by your liver. It forms part of the outer membrane surrounding every cell in your body. Cholesterol makes hormones, insulates nerve fibres and produces bile for digesting fat. You need cholesterol, but too much increases the risk of clots and heart problems.

Dr Ruben Mesa of the Mayo Clinic in the US explains: "There are two types of proteins called lipoproteins that transport cholesterol around your bloodstream. Low-density lipoproteins

(LDL) are also known as 'bad cholesterol'. They carry cholesterol to your body's cells. High-density lipoproteins (HDL) are also known as 'good cholesterol'. They remove excess cholesterol from the arteries, taking it back to the liver to be destroyed."

People with high LDL and low HDL are more at risk of heart disease, so managing cholesterol is particularly important for MPD patients. So what can you do?

**Get tested** You may not know you have high cholesterol as there are few symptoms. Ask your doctor for a test. This simple blood test will quickly indicate whether you need to alter your lifestyle or possibly, require medication to control your cholesterol.

## **Eat a healthy diet**

A healthy diet can reduce the level of bad cholesterol in your blood. Cut down on saturated and trans fats and replace them with monounsaturated fats and polyunsaturated fats.

Eating oily fish regularly can be beneficial as it provides the richest source of omega-3 fats, which are polyunsaturated

and can prevent the blood from clotting.

Soluble fibre can also help reduce cholesterol. This can be found in porridge, beans, pulses, lentils, nuts, fruit and vegetables. Whole grains are also proven to be better for your heart, so choose brown bread, rice and pasta.

The most concentrated sources of cholesterol include offal, egg yolk and whole-milk products. Instead, opt for lean cuts of meat, egg whites and skimmed milk.

## **Maintain a healthy weight**

Try to lose any excess weight. By shedding five to ten pounds you could significantly lower cholesterol. Adopting a healthy diet and a regular fitness regime may enable you to achieve your goal.

**Exercise** Whether or not you are overweight, exercise helps to control cholesterol. Moderate physical activity can increase good cholesterol, so consult with your doctor about a suitable fitness regime.

**Cut down on alcohol** Excessive consumption of alcohol

can also lead to high cholesterol, so drink in moderation.

**Stop smoking** As well as causing lung disease, smoking can raise your cholesterol and increase the risk of heart disease.

**Ask your GP** He or she can advise you on the ideal cholesterol levels for people with MPDs, who are at risk of blood clots. You can also learn more about managing cholesterol on the NHS website at [www.nhs.uk/livewell/healthyhearts/pages/cholesterol.aspx](http://www.nhs.uk/livewell/healthyhearts/pages/cholesterol.aspx)

**Ask your GP for guidance on medication** For certain people, lifestyle changes may not be sufficient to control cholesterol, and you may need treatment with medications such as statins. It is important to discuss this with your doctor. ■

## **Next Patients' Forum**

**London Nov 19th**

Find all the details on [www.mpd-support.co.uk](http://www.mpd-support.co.uk)

# When the goal is comfort, not cure

Ann Marie Jahn talks with oncology nurse Brenda Keenan on the misunderstood concept of palliative care

You may have heard the term “palliative care” and wondered what it meant. Brenda Keenan, a specialist in cancer nursing and a Certified Case Manager in the US, explains what palliative care is all about.

*Ann Marie Jahn: Palliative care has been in the press a lot lately. Can you explain a bit about how it works?*

Brenda Keenan: Some serious illnesses have limited treatment options. People who have these conditions can often benefit from palliative care. The goal of this care is not to cure the illness itself, but to maximise quality of life and comfort for the patient. This type of care is especially effective when patients have cancer or heart failure.

*So palliative care doesn't cure an illness. What sorts of problems does it address?*

People who have serious illnesses can suffer from many symptoms. They may feel fatigue, pain or shortness of breath. They may have lost weight, suffer from depression or be unable to sleep. Palliative

care addresses these and other similar problems.

*Is palliative care the same as end-of-life care or hospice care?*

No, it really is not. The main goal of palliative care is to help people live more comfortably with any chronic or life-threatening condition. Any time that patients are experiencing a diminished quality of life due to a chronic illness, palliative care can help.

*Who provides this kind of care?*

A team of medical professionals from several disciplines often works together to assist patients and families in alleviating suffering and improving quality of life. It's a comprehensive approach.

*What kinds of benefits does this care provide?*

There have been a few very interesting studies published about the benefits of palliative care. Some enlightening articles have appeared recently in US publications – the *New York Times* and the *New Yorker* magazine, available online. Research is



Brenda Keenan: maximising patients' quality of life

demonstrating that even patients who are very ill – in the late stages of cancer for instance – live longer and have a better quality of life when they have access to palliative care.

*Is this sort of treatment plan readily available to patients?*

Right now, palliative care can be difficult to access in both the UK and the US. In part, we need to think differently. Sometimes the best care is not the “cure” but living well with what you have. I feel positive that palliative care will become more widely available, because the benefits are clear.

*What can people do to relieve symptoms if they*

*aren't currently under palliative care?*

Patients and families have a definite role to play. If you or a family member are suffering from symptoms, you should keep a journal that records symptoms and notes the intensity of pain, shortness of breath, fatigue, constipation, nausea, loss of appetite, and difficulty sleeping.

Bring this journal to medical visits. Talk with your carers about how you feel – be honest and persistent and provide detailed information.

Complementary medicine can also provide solutions to seemingly intractable problems and is well worth a try. Ask your care team for advice and resources. ■



## Expert Briefing

Looking for medication options

**Q**I was diagnosed with essential thrombocythaemia (ET) six months ago. My platelet counts are over 1000. My haematologist advised me to begin therapy with hydroxycarbamide but I don't like the idea of taking that drug. Are there any alternatives? – BR

**A**You've recently been diagnosed with a chronic condition, so it's not surprising that you are uncomfortable with the idea of starting treatment. The key consideration when you have a myeloproliferative disorder (MPD) is to reduce your risk of suffering an adverse health event such as a blood clot. Haematologists can differ in when they prescribe treatment – some begin at 450 to 600 platelets and some at higher levels, up to 1500 for young patients who have never experienced a clot.

The decision depends on your overall health, age and whether you have suffered a clot before. Most people with ET tolerate hydroxycarbamide well in the low doses used to treat MPDs. Other drug options are available as well. Have an open dialogue with your haematologist to talk about your concerns and what might work best for you.

Best wishes,

– Dr Claire Harrison ■

*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.*

## When is aspirin most effective?

Dr Claire Harrison discusses whether aspirin is always beneficial

Despite landmark advances in our understanding of MPDs there have been relatively few significant advances in their treatment. What's more, there are sometimes conflicting opinions on treatment among medical professionals.

Young people and others who have low-risk essential thrombocythaemia (ET) often take low-dose aspirin as a way to prevent clots. A recently published research paper by Spanish scientists asks an important question: does low-dose aspirin in fact prevent clots as we have believed?

The authors of the publication looked retrospectively at the records of patients. They assessed how often clots occurred in 300 low-risk ET patients. These patients either received aspirin or were simply "observed" – in other words they did not take aspirin or other medications to treat their MPD.

The study authors report that overall rates of thrombotic events (clots) did not differ between these patient groups.

On the other hand, they also report that some patients – those who were JAK2 V617F positive and those who had cardiovascular risk factors – suffered more clots with observation only.

Patients with platelet counts over 1000 had an increased risk of major bleeding when they were treated with antiplatelet (aspirin) therapy.

The authors conclude that aspirin reduces the incidence of

venous thrombosis in JAK2-positive patients. It also reduces the rate of arterial thrombosis in patients with associated cardiovascular risk factors.

The authors also suggest that for other low-risk patients observation may be an adequate option.

Paradoxically, people with ET can have a tendency to bleed in addition to an increased risk of occlusive vascular disease. However, recent studies of low-dose aspirin (100 mg/day) in ET document its safety in low-risk patients and show that aspirin has the potential ability to reduce arterial thrombotic complications.

A recent review for both ET and polycythaemia vera (PV) concluded that the use of aspirin was associated with a statistically non-significant reduction in the risk of fatal thrombotic events without an increased risk of major bleeding, when compared with no treatment in patients with PV who have no clear indication or contra-indication to aspirin therapy. However, there was no adequate evidence for ET.

The results of the study from the Spanish study are provocative but somewhat inconclusive due to the way in which the study was structured.

Meanwhile a continuing prospective observational study in low-risk ET should further inform the question of safety and efficacy of low-dose aspirin in the setting of low-risk ET. ■

# Abseilers beat target, raising astonishing £74,000

Abseil organiser Rachel Bridgman explains how MPD Support's abseiling team reached summit of success

Last year I made myself the promise that I would raise as much money for MPD Voice as I could by the end of 2010. Early this year I was still looking for ideas and I hadn't come up with any good solutions. Then someone suggested an abseil.

That's not such a good idea, I thought, because I'm afraid of heights. In fact I once experienced vertigo at the top of my children's climbing frame – and on that occasion I needed to be “talked down” by my twelve-year old. There was no way I planned on

with myeloproliferative disorders to consultants, nurses, friends and family.

Everyone who participated raised substantial sums for research, and what's more they accomplished the feat of abseiling down the world's highest hospital tower! We had plenty of supporters making lots of noise as well – children helping and a South American band playing music. The sun shone all day.

I had crossed my fingers and hoped that we could all raise £20,000. I continue to be astounded at the total of £74,000. It seems an unthinkable high amount, but it also reflects my belief that people wish to support the work of MPD Support. I am incredibly proud of all the abseilers and awed at the generosity of our donors.

I look back on the day of the abseil as an important one for me personally. I made lots of new friends, but the most important



Celebrating abseiling success: Hazel Butler (left) and her daughter Jill Owen (right) together raised over £15,000

to me are my new MPD Voice friends, and I am really looking forward to seeing them again.

A powerful mix of adrenaline, excitement and positivity carried the abseilers and their supporters through the day and made it one of the most exciting days of my life. ■



Peter Heath and Rachel Bridgman

dangling off the outside of a very tall building on a very thin rope. But what better way to spread the word about fundraising – I would ask other people to do it for me!

I worked with MPD Support's chairman Dr Claire Harrison and together we found a team of fantastically brave abseilers. They were a completely mixed bunch, including everyone from people



Scaling new heights, from left: Stephen Cunningham, Neil Lipscomb, Jonathan Baker, Tim Farnell-Watson, John Harley and Sarah Wilford



# Cyclist with PV tackles Alpine passes

John Taylor and his team take on a fundraising and cycling challenge

My name is John Taylor and I was diagnosed four years ago with polycythaemia vera (PV). I have been a keen cyclist all my life, so when I was diagnosed I was determined not to let this disease stop me riding.

Learning I had PV was quite a shock. In the early days right after my diagnosis I was crushed by fatigue. I had to fight to regain my fitness level, but I kept cycling with encouragement from my wife and my cycling mates. The physical and mental energy I derive from this sport has helped me deal with polycythaemia vera in a positive way.

It's been a few years since I began treatment, and I now feel confident and fit enough to do something to help MPD Support. I decided to organise a fundraising cycle ride in the French Alps.

My cycling friends and I will tackle some of the highest peaks in the Alps to raise funds for MPD Support. We'll attempt

to climb as many high Alpine passes (or *cols* as they are called in French) as possible in our Col Conquering Challenge – in fact over a two-day period we'll be climbing as many as in the Tour de France.

This will be a really difficult challenge as some of these cols climb to 6000 feet and more. We'll be riding straight uphill for eight to twenty or more kilometres with an average gradient of ten per cent on each col.

I'll consider it a success if we conquer just one col. It's tough for anyone but even tougher with polycythaemia vera. I'll be tackling the Alps with friends – our average age is 59 years. Luckily none of my friends has an MPD, but given our ages we each have our own health issues.

If we can do this it proves that anything is possible, even with diseases and age against us. Please sponsor us – visit [www.mpd-support.co.uk](http://www.mpd-support.co.uk) and click on the link from the homepage. ■



Alpine team pictured left to right: Derek Woodings, Bill Cotton, Brian Newton, Alan Hitchcock and John Taylor.

Andy Swinburne (not shown) will also attempt the Col Conquering Challenge

## Our next issue

- An expert in caring for teenagers with cancer discusses the issues facing young people with MPDs
- Complementary therapies: value that can be proven
- How do haematologists define “low risk”?

*mpd-support*

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