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Let the patient revolution begin

Patients can improve healthcare: it’s time to take partnership seriously

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A hundred years ago George Bernard Shaw lambasted the medical profession as a conspiracy against the laity.1 Today, disease and doctor centric health systems that are costly, wasteful, fragmented, and too often uncharing are provoking similar ire.2

Despite the best intentions and undoubted skill of many who work within healthcare, access to care, and its quality, vary markedly, and most people in rich countries access a confusing smorgasbord of tests and treatments whose merits are hyped and harms underplayed.3 Patients lack information on practice variation, the effectiveness of their care, and the extent of medical uncertainty. Practice is informed by an incomplete research base bedevilled with selection and reporting bias,4 and at worst fraud. The preservation of institutional bureaucracies, as well as professional and commercial vested interests, have consistently trumped the interests of patients. The healthcare industrial complex stands accused of losing its moral purpose.5 This corruption in the mission of healthcare requires urgent correction. And how better to do this than to enlist the help of those whom the system is supposed to serve—patients? Far more than clinicians, patients understand the realities of their condition, the impact of disease and its treatment on their lives, and how services could be better designed to help them.6

Clinicians and patients need to work in partnership if we are to improve healthcare and challenge deeply ingrained practices and behaviours. Doing this won’t be easy for either side after eons of paternalism, and many patients will prefer to maintain the status quo. But good examples are showing the way. The Choosing Wisely initiative in the US (www.choosingwisely.org/) brings patients and doctors together to identify and reduce the use of unwarranted and ineffective interventions. Discussion groups of patients, carers, and clinicians led by the James Lind Alliance in the United Kingdom, and the Patient Centered Outcomes Research Institute in the United States, are shedding light on the mismatch between the questions that patients and doctors want answers to and the ones that researchers are investigating. Joint discussions have helped build a database of uncertainties about the effects of treatment (www.library.nhs.uk/duets/).

Patients and doctors are also collaborating to design new services and information systems.7 Leaders in innovative partnership include ReshapeHealth (www.radboudreshapcenter.com), which is pioneering patient led and “crowdfunded” research. A growing number of healthcare organisations are giving patients access to, and in some cases control over, their medical records.8 At the Mayo Clinic a free app gives patients full access to their medical notes, pathology reports, and radiology reports; and because a shift in power depends on establishing a common lexicon, work is under way to reduce the medical jargon in these resources. There are guides on why and how to engage with patients (http://epatientdave.com/let-patients-help/),9 and some patients are already acting as “sherpas” to promote joint working,10 including members of the participatory medicine movement (http://participatorymedicine.org).

Online patient communities where patients meet, talk, support, inform, and coach each other are empowering patients (although it is important to note who sponsors them).7 They also provide a rich and as yet largely untapped learning resource for health professionals. Examples include healthunlocked.com, healthtalkonline, rawarrior.com, and cancergrace.org (www.bmj.com/podcast/2013/04/29/dying-patients-hospital-e-patients-online). There are salutory lessons in the gulf between conversations in the clinic and the concerns patients share with their peers.

Advocacy for patient engagement in the US, UK, mainland Europe, and well beyond is driven largely by the belief, backed by some evidence, that engaging patients will reduce healthcare costs through the avoidance of unnecessary investigation and treatment. Patient engagement is seen as a way to help health systems become sustainable. Some have argued that it is the “blockbuster drug of the century” and will deliver equivalent dividends.10

But partnering with patients must be seen as far more than the latest route to healthcare efficiency. It’s about a fundamental shift in the power structure in healthcare and a renewed focus on the core mission of health systems. We need to accept that expertise in health and illness lies outside as much as inside medical circles and that working alongside patients, their families, local communities, civil society organisations, and experts in other sectors is essential to improving health. Revolution requires joint participation in the design and implementation of new policies, systems, and services, as well as in clinical decision making.

Much remains to be discovered, evaluated, and implemented to achieve meaningful partnership with patients. There is also a need to embed shared decision making into routine practice.11,12 At an open meeting in June in Peru (www.isdm2013.org), which can be followed through social media, the shared decision making community will further global debate on the latest thinking and research.

For its part the BMJ is stepping up its commitment to patient partnership. We already have an online collection of articles on shared decision making and a growing library of patient journey articles.13 Now we want to develop a strategy for patient partnership that will be reflected across the entire journal. We plan to establish a panel of patients and clinicians to help us with this work and will report back on our progress.

It has been said that healthcare won’t get better until patients play a leading role in fixing it.14 We agree and look forward to helping drive the patient revolution on.

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*ANALYSIS, p 21; OBSERVATIONS, p 25; PERSONAL VIEW, p 27*
Should we be reassured about sitagliptin?

Remember that the absence of proof of harm is not proof of absence of harm.

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New drugs for the treatment of diabetes have had to fight hard to prove their safety. The latest class of drugs causing concerns is the dipeptidyl peptidase-4 (DPP-4) inhibitors. Current evidence suggests that these drugs increase the risk of pancreatitis and they are undergoing close study for evidence of sufficient safety to warrant their continued use.1 In a linked paper, Eurich and colleagues contribute to the growing evidence base regarding sitagliptin and related drugs and provide some evidence of their safety.2

Sitagliptin was approved by the US Food and Drug Administration (FDA) in 2006 as the first in a new class of antidiabetic drugs, the DPP-4 inhibitors. It was approved for use as monotherapy or combination therapy, with diet and exercise, for treatment of type 2 diabetes. During the first three years after approval, the FDA received 88 case reports of acute pancreatitis. This prompted a revision of the prescribing information in February 2009 to include recommendations that prescribers monitor patients for signs and symptoms of pancreatitis at initiation of this drug and at dosage changes. Other labeling revisions followed, including warnings about hypersensitivity reactions and acute renal failure. In March this year the FDA announced that it is actively evaluating the risk of pancreatitis and pre-cancerous findings of the pancreas from incretin mimetic drugs for type 2 diabetes.3

Eurich and colleagues conducted a retrospective cohort study of patients receiving glucose lowering drugs, using an integrated claims and laboratory database. They found no increased risk of hospital admission, all cause mortality, or death from cardiovascular disease in patients newly exposed to sitagliptin relative to other glucose lowering drugs. They rigorously controlled for the confounding that would be introduced by naively comparing people exposed to sitagliptin with those who were not exposed. The authors had hypothesized that users of sitagliptin would not have more hospital admissions, all cause mortality, or cardiovascular events than those not using sitagliptin. These endpoints were chosen because of their importance to patients and clinicians, and not because signals to date suggest cardiovascular risk. On the contrary, the authors noted that pooled analyses of clinical trials had suggested cardiovascular benefits with DPP-4 inhibitors.4

The authors made many smart analytic choices, including the evaluation of incident users of the drug, careful control for confounding by indication, and ample sensitivity analyses where they varied the choices made in the primary analyses. However, it may be hard to interpret these results in light of their hypothesis of no increased risk. The absence of proof of harm is not proof of absence of harm.5 The authors do not describe the study’s adequacy to demonstrate small increases or decreases in mortality, hospital admissions, or cardiovascular events associated with the drug. As with equivalency trials, a retrospective cohort study that is designed to show an absence of difference between exposed and unexposed people should state what minimally important difference would be detectable in the cohort and what difference may be clinically meaningful.6

Why did Eurich and colleagues not see an increased risk of pancreatitis? The absence of a signal for what is now widely recognized as an outcome that can be attributed to this drug is surprising.7 They report an adjusted hazard ratio of 1.10 (95% confidence interval 0.68 to 1.77) for acute pancreatitis. The absence, in this study, of an association between sitagliptin and acute pancreatitis can be interpreted in two ways: either the study adequately controlled for the confounding that has plagued other studies or the study was underpowered for this outcome and possibly the primary outcomes of interest too.

Eurich and colleagues’ cohort included more than 8000 people exposed to sitagliptin. The mean exposure time to an antidiabetic drug was 2.5 years. This is longer than the follow-up times in the clinical trials that led to the approval of such drugs that were subsequently included in meta-analyses, but it is still not a long time in the life of patients with diabetes. However, for events that are not expected to be associated with the drug (death from cardiovascular disease and all cause mortality), the number of exposed people may have been too few to detect a significant increase in risk. In meta-analyses to date, data were included from 20 312 people exposed to DPP4-inhibitors (not just sitagliptin).

We can certainly admire and learn from the careful methods used in this study, but we should remember that additional considerations may be needed upfront in trials that pose a null question. Perhaps observational studies should be designed like non-inferiority trials, with prespecified minimal detectable differences between groups. Although this study contributes to the evidence base about sitagliptin, it is unlikely to change practice, the current investigation of the safety of DPP4-inhibitors, or regulations.

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Growth factors present in blood products... is thought to improve the healing process in chronic injuries and to accelerate repair in acute and chronic lesions

AutoLOGous blood products in musculoskeletal medicine

Although they are trendy money spinners, best evidence shows little effectiveness

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Participation in almost all sports and physical activities benefits individuals and society as a whole because it promotes health and helps prevent conditions such as osteoporosis, cardiovascular disease, and diabetes; it may also lead to improved mental health. Nevertheless, athletes are highly vulnerable to musculoskeletal injuries, sometimes with devastating effects. Sports injuries cost society billions of dollars in direct and indirect costs. Regenerative medicine technologies, such as autologous blood products for the treatment of tendinopathies, hold the promise of improved outcomes for musculoskeletal conditions that currently have limited or no treatment options. In a linked trial, Bell and colleagues compare the effectiveness of peritendinous injections of autologous blood with standard eccentric exercise in athletes with Achilles tendinopathy and find no difference.1

Novel treatments that are reported to accelerate recovery from musculoskeletal injuries, without adversely affecting recurrence rate, are increasingly advocated in the lay media and often heavily promoted to athletes and healthcare professionals.2 3 Injections of autologous blood products, including whole blood, platelet rich plasma, and autologous conditioned serum, are increasingly used in the management of sport injuries, yet they are supported by a poor evidence base.4 Why are blood products appealing? Blood contains biologically active components responsible for haemostasis and can potentially initiate synthesis of new connective tissue and promote revascularisation.5 Growth factors present in blood products, and the potential of these growth factors to induce further release of such factors, is thought to improve the healing process in chronic injuries and to accelerate repair in acute and chronic lesions. Treatment with platelet rich plasma, in particular, is increasingly thought to accelerate muscle and tendon healing and to allow early return to competition in elite athletes. It is therefore often recommended as best practice for management of musculoskeletal injuries. The global market for this product, valued at $4.5m (£2.9m; €3.4m) in 2009, is expected to be worth more than $120m by 2016.6

Although autologous blood injections are routinely performed, many unanswered questions remain, including the most appropriate volume and frequency of injection, the ideal time between injections, and the mechanism by which the beneficial effect is harnessed.7 8 These treatments for musculoskeletal injuries have been tested in few well conducted randomised controlled clinical trials, and their optimal application is unclear. A recent systematic review showed that, when these products have been tested in appropriately powered studies, with strict outcome measures, by researchers with no conflicts of interest, the results have been underwhelming and would not justify the use of such products over more traditional treatment.9 Nevertheless, many experts swear by such treatments, and many athletes, coaches, managers, and agents would not forgo these interventions after musculoskeletal injury.

Bell and colleagues’ study adds to the small evidence base in this field.1 Achilles tendinopathy is prevalent and debilitating, being common in athletes and the sedentary population. Indeed, it is often used as an example of troublesome chronic and recurrent soft tissue injury. The authors compared injection of whole blood with stable conservative management—eccentric exercises (although it is unclear whether these exercises are effective) and used appropriate outcome measures in an adequately powered study. Advocates of blood product treatments are likely to criticise the study on several grounds. Patients were enrolled after experiencing only three months of symptoms. However, it may be argued that from a biological viewpoint, the typical tendinopathic lesion, which represents a failed healing response, is already chronic at clinical presentation.10 The injections were unguided because the authors wished to reproduce the clinical situation of a clinician with no access to ultrasound guidance. Only two injections were performed, whereas many advocate at least three, although it is unclear whether three injections are better than two.

Other critics may say that it is not surprising that whole blood is ineffective and that platelet rich plasma should have been used. However, the only randomised controlled trial on the use of platelet rich plasma in Achilles tendinopathy found it to be no more effective than standard treatment.11 Furthermore, the results of a recent randomised trial that directly compared injections of platelet rich plasma and autologous whole blood indicated that whole blood was slightly superior at six months in patients with refractory elbow tendinopathy.12 It may be that not all tendons are the same and that, for example, the elbow extensor tendons are more amenable to blood product injections than tendons in other locations.13 However, even in the upper limb it is becoming apparent that autologous blood products, when tested in randomised controlled trials, are no more effective than standard, less expensive, and less time consuming care.14

Clinicians and patients often want a magic bullet, and autologous blood products seem to satisfy many requirements of different parties. They are easy to obtain and prepare, they seem to be safe and ethically and socially acceptable, they are not doping, they are hyped by the media, and they allow large profit margins. But do they actually work? Despite the findings of well conducted studies that suggest that they probably do not, it seems likely that they will continue to be widely used.

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Peritendonous injections of blood products are already in widespread use


Implications of universal screening for HIV infection

Ethical concerns must be considered and sound practices adopted

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Universal voluntary screening of all adolescents and adults for HIV infection is now recommended by the United States Preventive Services Task Force.\(^1\) This follows similar recommendations announced by the US Centers for Disease Control in 2006.\(^2\) Although there are good reasons for endorsing universal screening, the ethical and practical implications of this approach need to be considered.

Universal voluntary screening could help identify asymptomatic people who are infected with HIV but who might otherwise go undetected until late in the course of infection. It is essential to identify such people for their own personal health and the health of others to whom they may unwittingly transmit the virus. Because of advances in the care of HIV infection, early treatment is paramount, both for individual benefit and for decreasing the likelihood of transmission. Taken together, there is a clear public health imperative to screen for this infection.\(^3\)

A crucial aspect of the task force’s recommendations is that universal screening is done with notification and that patients can “opt out” of being tested. It is also recommended that before testing patients are counseled about the meaning of a positive or negative test.\(^4\) Opting out, rather than opting in, is designed to encourage uptake of testing. This approach is very different from the elaborate counseling and testing, typically accompanied by written informed consent, as required by multiple statutes across the US, which is seen by many as a barrier to testing.\(^5\) Although there were sound reasons for this practice, which was developed early in the AIDS epidemic when treatment options were limited or non-existent and HIV related stigma was widespread, now that good treatment options exist, some question its value. After all, a simpler model is akin to most routine laboratory testing.

Nevertheless, although the process for arriving at agreement to undergo HIV testing should be streamlined, the accompanying counseling is important in some circumstances. Counseling can comprise more than a discussion of the implications of test results. For example, counseling provides an opportunity for discussing ways to prevent HIV infection among those at high risk (such as pre-exposure prophylaxis) as well as safer sex practices that can help mitigate the likelihood of acquiring HIV and other sexually transmitted infections. For those who are found to be infected, counseling on the finding and its implications is crucial for ensuring physical and psychological wellbeing, especially for those who are not familiar with the implications of being infected.

Unfortunately, because proper counseling requires time and expertise, it may be difficult to implement in the clinic. With office encounters limited in time and replete with other competing priorities, clinicians will need to find efficient ways to provide patients with relevant information about the many effective methods of prevention and treatment. For example, it may help focus the conversation if patients are sent information about HIV before their appointments. In addition, because discussing HIV testing can also open the door to important questions about sexual and other risk behaviors that warrant close attention, practitioners in general clinic settings will need to develop relevant expertise in counseling on these issues.

The history of the HIV epidemic reminds us of the need for ensuring confidentiality as screening becomes widespread. In the past, just being tested could result in discrimination with regard to employment, housing, and insurance. General provisions for the privacy of medical information exist,\(^6\) and information on genetic testing has some special legal protections in the US.\(^7\) However, HIV testing does not currently benefit from such special provisions.

HIV infection remains stigmatized in some settings. The introduction of screening may reduce stigma, but in the short term patients’ concerns about stigma need to be taken seriously to ensure that they are not inadvertently harmed. Accompanying universal screening with widespread public health programs on the nature of HIV infection might help chisel away at HIV related stigma.

Part of the justification for identifying people with HIV infection rests on the safety and efficacy of current treatment approaches. Although some of those identified will have reasonable access to good treatment, it will be crucially important to ensure proper linkage to care in community settings.\(^8\) It is essential that care is made available to those who need it, and ensuring that they receive it will require sound approaches to delivering treatment.

Universal screening for HIV infection is a crucial step in helping to curb the epidemic. Knowledge about HIV status ultimately is power for those who are found to be infected. However, to achieve the goals implicit in the new recommendations for universal screening, those implementing such screening must be sensitive to the related ethical and practical challenges.

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