Introduction

In the United Kingdom, the pharmaceutical, medical technology, medical and industrial biotechnology sectors generate a turnover of over £50 billion and employ 166,000 people in 4,500 companies. The pharmaceutical sector is the largest contributor to turnover and employment (388 companies, 78,000 employees and £31.8 billion), followed by the medical technology sector (3,130 companies, 64,000 employees and £15 billion).¹

The cost of each element of a health service will always be a concern for the governments that support it and medicines are usually the first element of spend to come under scrutiny, probably because of the relative ease of applying pressure, when compared with the cost of manpower and infrastructure. The UK National Health Service (NHS) spends around £10 billion a year on branded prescription medicines in the United Kingdom (approximately 10 per cent of the healthcare budget).² While the total spend on medicines will continue to increase by a forecast 3.5 per cent CAGR (Compound Annual Growth Rate) from 2011 to 2015, the growth rate for branded medicines is forecast to be just 1.1 per cent CAGR over the same period.

Interestingly, a 2010 comparison of the prices of branded medicines in the United Kingdom with prices in a range of European countries and the United States and Australia for 2010 shows that the United Kingdom is among the lowest compared to other European comparator countries.²

As to how companies interact with the DH on pricing, there are two alternatives: the voluntary scheme, the Pharmaceutical Price Regulation Scheme (PPRS), and a set of statutory regulations. It is important to note that technically, for new chemical entities, freedom of pricing remains in the United Kingdom. For branded non-NCEs, the Department of Health (DH) reviews and negotiates price, and for generics the drug tariff applies. This means that, in theory, pricing and reimbursement are two separate processes in the United Kingdom, where price setting is a matter reserved for the UK Government and administered by the English DH, while practical uptake or reimbursement is largely a matter for NICE in England and Wales, AWMSG in Wales where no NICE guidance exists, and SMC in Scotland.

The voluntary scheme, the PPRS, was first established in 1957 and since then has been the established mechanism by which the DH (on behalf of the UK health departments) seeks to achieve a balance between NHS access to good quality medicines and the need to control costs. The scheme operates through a process of annual negotiations, where companies and the DH meet to agree prices for new and existing products. The scheme is governed by a framework document, the PPRS, which sets out the principles and procedures for negotiations. The scheme operates on a voluntary basis, with companies choosing to participate or not.

branded medicines at reasonable prices and a fair return for the industry to enable it to research, develop and market new and improved medicines. The key advantage of the voluntary scheme is a five-year period of predictability in the market, as it is renegotiated every five years, whereas the statutory regulations may change at any time, subject to approval by parliament.

Up to the beginning of 2012, 167 companies were signed up to the 2009 scheme. The 64 companies that did not join were subject to statutory controls under the Health Service Branded Medicines (Control of Prices and Supply of Information) (No.2) Regulations 2008 (‘the Statutory Scheme’).

In addition, consideration was given around the same time to establishing a value-based pricing (‘VBP’) scheme (now known as Value Based Assessment (‘VBA’)), that as proposed would have had the NHS (through the National Institute for Health and Care Excellence (NICE)) dictating prices to companies – as opposed to deciding whether or not to accept the companies’ prices. The change had concerned some in the sector, which is already under serious pressure to cut the cost of medicines in the face of the European financial crisis.

The PPRS

On 3 August 2012, the Association of the British Pharmaceutical Industry (ABPI) and the DH published a joint DH/ABPI statement on arrangements for pricing branded medicines from 2014.

Pricing negotiations commenced in September 2012 and were set to conclude approximately a year later. These negotiations would determine the arrangements for pricing branded medicines in the United Kingdom from January 2014, including medicines that were already on the market in December 2013 and new medicines launched from 1 January 2014.

The joint statement began as follows:

The Department of Health and the Association of the British Pharmaceutical Industry (ABPI) are committed to reaching agreement on a pricing system that gives patients better access to the most effective medicines, at prices that encourage the NHS to use those medicines when clinicians think their patients can benefit and deliver value to the NHS, and provide a fair reward for these innovative medicines.

When the current Pharmaceutical Price Regulation Scheme (the 2009 PPRS) comes to an end in December 2013, we will move to new arrangements which will incorporate a broader assessment of value of a medicine, known as value based pricing, for new medicines (new active substances), in conjunction with a successor scheme to the 2009 PPRS.

The current branded medicines pricing scheme, the PPRS, is a voluntary scheme agreed between the Department of Health and the ABPI as the recognised body representing the branded pharmaceutical industry.

Our joint aim is to achieve a negotiated agreement for the new arrangements, including value based pricing. We expect negotiations will begin later this year and these will cover both value based pricing and the successor scheme to the 2009 PPRS. In addition to the voluntary arrangements that we hope to agree through negotiation, there will continue to be a statutory scheme for those companies that choose not to participate in the voluntary arrangements.

On 6 November 2013, the DH announced that the ‘heads of agreement’ of the voluntary PPRS had been agreed between themselves and the ABPI, acting on behalf of the pharmaceutical industry. The headline of the agreement was that the industry would not face the now traditional ‘up-front’ price cut but that a payment will be calculated based on NHS net sales based over and above an overall drugs bill cap. This was intended to be a one-off contribution to ‘austerity’, after which it may ‘get back to normal’ from the end of 2018.

The growth caps are set out below, as are the estimated payments back to the UK DH required of the industry (subject to some exceptions); 2014 is set at a specific level but thereafter it will be based on actual increases in the NHS drugs bill.

The exemptions include the fact that new chemical entities (‘NCEs’) are exempt from the manufacturer’s rebate calculations but will be included in the overall drug bill growth
calculations. This effectively means that established products, in line with previous years’ PPRS schemes, pay for future market entry and growth of new products. It is important to understand that medicines that are not classed as NCEs, based on DH seeking Medicines and Healthcare Products Regulatory Agency (MHRA) confirmation, are neither exempt from the PPRS payments, nor do they enjoy freedom of pricing. Therefore, significant innovations involving new uses for old compounds or examples of ‘incremental innovation’ are afforded no incentives under the PPRS.

There are certain companies that will be exempt from the payments, in particular companies with £5 million net NHS sales. However, there is no exemption for the first £5 million for those companies with sales in the range £5 million to £25 million as there was in the previous scheme.

The official statement provides more detail as well as web links to the full PPRS agreement.³

The Statutory Scheme

The DH launched a consultation in June 2013 outlining planned revisions to the Statutory Scheme. It proposed 10 per cent, 15 per cent or 20 per cent price reductions for the medicines the NHS purchases through the scheme and 15 per cent was finally settled on as the DH’s preferred option.⁴ It was estimated that this would achieve a net benefit of £1.256.8 million.⁵

The consultation also proposed that the Statutory Scheme, like the PPRS, should apply to average selling prices (‘ASPs’), which are the prices negotiated by hospitals. These negotiated discounts typically exceed the price reductions required under the Statutory Scheme; therefore, adjusting prices in the Statutory Scheme alone may not reduce the price the NHS is actually paying. However, it was decided in the final regulations that the 15 per cent price reduction would not apply to ASPs as this was deemed to be too complicated and required further consideration. Future consideration by DH on this issue may result in the price reduction being applied in addition to any discounts already negotiated and reflected in the ASPs. This remains one of the key areas of uncertainty for companies subject to the Statutory Scheme.

In addition, the final Statutory Scheme included the removal of certain exemptions, replacing them with one to protect small firms (those with NHS sales of branded medicines of less than £5 million per annum) from the price cut and information provisions. The final Statutory Scheme also revises the information-gathering requirements, so that necessary information is collected and may enable the DH to apply the price cut to ASP in the future.

Beyond the headline changes there could be some other issues to bear in mind when considering the Statutory Scheme:

- The Statutory Scheme refers to the principle that there should be observance of not just the letter of the law but its spirit; one hopes that this principle would not just apply to companies but that the DH would act similarly and be flexible, for example in its leniency to small and medium-sized enterprises (SMEs).
- There is no clear appeal process suggesting that judicial review is the only recourse against an unfair decision.
- Penalties will apply to UK health service sales when ‘necessary information’ is not provided.

How has the PPRS Deal Affected Value-based Pricing?

The plan had been to develop reforms to the way value is measured, in parallel with the negotiations on the PPRS.

The current methodology used by NICE to appraise new technologies including drugs is the calculation of additional health benefits, measured in QALYs (Quality Adjusted Life Year) per unit cost of the new intervention, compared with existing treatment.

In June 2013, the DH provided NICE with terms of reference for the development work to support value assessments in the context of VBP being the then-favoured approach. These stated that the methods for value assessment of branded medicines under VBP should:

- be applied to medicines within the scope of the VBP system, and incorporated into the methods for other categories of guidance at NICE’s discretion;

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● adopt the same benefit perspective for all technologies falling within the scope of VBP, and for displaced treatments;
● be as transparent and predictable as possible;
● be informed by the best available evidence;
● include a simple system of weighting for burden of illness that appropriately reflects the differential value of treatments for the most serious conditions;
● encompass the differential valuation of ‘End of Life’ treatments in the current approach within the system of Burden of Illness weights;
● include a proportionate system for taking account of Wider Societal Benefits;
● not include a further weighting for Therapeutic Innovation and Improvement;
● produce guidance for patients and the NHS which describes the clinical and cost effectiveness of the technology and its position in clinical practice.

VBP has clearly not happened, with the concept of having NICE setting prices under the VBP concept seemingly abandoned and VBA now likely to be delayed until late 2014, nearly a year after the new pricing agreement came into effect.

On 6 January 2014, NICE launched a consultation on the guide to the processes of technology appraisals (this considers exactly what happens and when, throughout technology appraisals), which was open until 28 March. This consultation does not address the methods of technology appraisals (such as the consideration of burden of illness and wider societal impact), which are the subject of a separate consultation after the NICE Board approves the relevant proposals. At the NICE board meeting on 22 January 2014, papers were presented to the board for their approval, beginning the process of a public consultation on the NICE methods to assess health technologies. These papers laid out the draft proposals, which appeared to water down the original intentions of government further still by offering an alternative to wider societal benefits (‘WSBs’) that were originally proposed as the key new criterion for value assessment. Instead, wider societal impact would be assessed using the absolute shortfall in QALYs resulting from living with a disease or condition. NICE states that this alternative approach may not be fully consistent with the terms of reference, but the institute believes that its merits should be considered alongside those which accompany the approach explored by the DH.

NICE is quite clear that any approach to wider societal benefit will inevitably take age into account. The age of any individual has an impact on what they are able to contribute to, and consume from society. They state that ‘it would be quite wrong for NICE to use the simple fact of the age distribution of people with particular conditions as the basis for deciding whether or not the NHS should offer new treatments, just as it would be wrong to use gender or any of the other “protected” characteristics under the equalities legislation.’

On 9 January 2014, NICE responded to a press article on the topic of their changing methods. Sir Andrew Dillon, Chief Executive of NICE, said: ‘We have no intention of introducing a change to our methods that would disadvantage older people.’

This is somewhat reassuring in the sense that future legal challenges based on protected characteristics are likely to be fewer, but it does suggest that any new value-based assessment of technologies will not be dissimilar to what we have today. In fact, what this appears to mean in practical terms is that the NICE process will be largely ‘business as usual’ and, as has been the practice in the past, the QALY will be at the heart of every decision.

However, the threshold that NICE applies to determine whether a medicine is good value for money may be changing. In the existing methods guide (2013), manufacturers are asked to present the probability that the treatment is cost effective at maximum acceptable ICERs of £20,000 to £30,000 per QALY gained. In the latest proposals from NICE, they state that the threshold at which a technology is judged to be good value for money for the NHS is currently £20,000 per QALY gained. Is this a reduction to the threshold by stealth, or a genuine oversimplification by NICE?
UK Pricing and Health Technology Assessment: the International Dimension

Medicine prices differ across the European Union (EU) due to factors that are often beyond the control of companies. In Europe price is decided at a national level; it is not an EU competence. National health and pharmaceutical policies and priorities, wholesaler and pharmacy margins, VAT rates, pack sizes, distribution channels and exchange rate fluctuations all influence price in any individual EU Member State. There is an EU Pricing Transparency Directive but in broad terms it only covers the requirement for national pricing and reimbursement decisions to be open and objective, to be within stated timelines and reviewable, and for there to be an appeal mechanism. It is currently under review.

Within Europe, Member States compare and sometimes reference their prices to other countries’ prices. Reference pricing is very complex, and to avoid unanticipated results the relevant authorities need to take account of differences between Member States such as purchasing power, GDP per capita, country-specific pharmaceutical regulation and policies, and also countries under austerity measures, especially where temporary measures have been introduced to adjust price.

Lower prices through reference pricing can force companies to consider the impact of launching a product in one country on the revenues of other countries. Ultimately, when a country’s international reference pricing (‘IRP’) policies are extremely aggressive, such as when they base their price on the lowest price in a range of countries in a ‘basket’, it directly impacts the profitability of launching a product in that country.

The lack of a headline price reduction under the PPRS (as opposed to under the Statutory Scheme) shows that the DH has been persuaded at least of the influence of UK prices on IRP. However, it does overlook the reality of the situation that discounts, rebates and underlying ‘net’ prices, are taken into account by some Member States, such as the AMNOG process in Germany.

Impact on the Life Sciences Industry

In his 2013 Autumn Statement, George Osborne (UK Chancellor of the Exchequer) announced that a new science and innovation strategy would be produced in time for the 2014 Autumn Statement and would include plans for spending on new infrastructure. He said: ‘To ensure that UK capabilities remain world-leading in the long term, the Government will produce a Science and Innovation Strategy for Autumn Statement 2014’, and ‘Central to this will be a roadmap of how the Government’s long-term commitment on science capital announced at Spending Round 2013 will deliver the research and innovation infrastructure needed to ensure that the UK’s capabilities remain world-leading while playing a key role in economic growth and scientific excellence.

A recent review of the current Strategy for UK Life Sciences commented that real progress had been made over the past two years, which has had a positive impact on the life sciences industry. This is clearly positive for the United Kingdom, but it is important that the good work done is not undermined.

Erosion of pricing and its effect on industry is difficult to simply quantify because of the multiple policies and stakeholders involved in the process of taking an innovation from laboratory bench to the patient’s bedside.

However, it is common sense to say that lower prices decrease incentives and the ability to innovate, and, perhaps more relevant for patients served by the UK NHS, to launch medicines in a given country. Developments such as these new UK price reductions not only impact the revenues of pharmaceutical companies, but can also act to delay patient access to new innovative treatments.

Innovative companies must constantly adapt to take into account the range of schemes operating in different countries, from schemes focusing only on affordability or cost-effectiveness to those that do not constrain the rewards so as to ensure patient access to highly innovative treatments.

The European School of Management and Technology produced a White Paper in 2009, which concluded that, in designing optimal pharmaceutical pricing and reimbursement regulation, the benefits of more affordable or cost-effective drugs must be traded against the costs of less pharmaceutical innovation, with fewer projects being developed in general and in particular in low-margin therapeutic areas and with little potential of being considered highly innovative at the time of market launch.14

Finally, it is important to consider how the new regime might impact investment in stratified or personalised medicines such as highly effective biotechnology therapies. Such therapies involve the same or higher development costs in smaller populations and typically have a higher unit price. Manufacturers may be understandably worried about the likelihood of achieving acceptable levels of pricing and then uptake through what appears to be an unpredictable route to market in the United Kingdom.

**Conclusion**

It is certainly a very difficult task to reach a long-term successful balance between facilitating NHS access to good quality branded medicines at reasonable prices and a fair return for the industry to enable it to research, develop and market new improved and innovative medicines.

Will this latest round of negotiation between the DH and ABPI be seen as a success or failure? The answer must be judged over time against a range of measures, including the level of inward investment by life science companies, launch sequencing in the United Kingdom compared with our neighbours in Europe, and the total size of the branded medicines bill and the corresponding size of the repayment to the DH.

Our initial view is that these new arrangements will stifle inward investment into the United Kingdom as companies find it increasingly difficult to justify investment decisions based on lower net UK prices when compared with other global markets.

In its Plan for Growth and its Strategy for the Life Sciences, the UK Government has stated its support for an early access plan (to new innovative technologies) and there are growing rumours of support for an innovative medicines bill, which may or may not come to fruition.

Whatever impact the outcome of the recent pricing negotiations may have, there is the possible unintended consequence of limiting UK patients’ access to new innovative medicines, as companies, particularly innovative small and medium-sized enterprises (SMEs), delay launch in the United Kingdom while they negotiate a more favourable unit price elsewhere in Europe. There are clearly short-term financial benefits for the United Kingdom in this new deal, but these benefits may lead reduced patient access and the financial consequences to health and ultimately finances in the longer term. It remains to be seen if the DH and NICE will apply a pragmatic approach to individual cases to reduce the risks to access. In addition, only time will tell how UK pricing policy interrelates with, and impacts on, the government’s commitments in its UK life sciences strategy.

The latest round of pricing negotiations leaves open many questions. We have highlighted some of the outstanding issues below to assist innovators as they consider how future pricing decisions in the United Kingdom may impact important strategic decisions including research and development investment and launch strategy.

- How will the PPRS and value-based assessment impact pricing of medicines under early access schemes, assuming they are a new chemical entity? Will existing patient access schemes be in danger as manufacturers realise they have to reduce the price further than originally negotiated with the DH and NICE?

- How will UK pricing changes impact the take-up and investment in the United Kingdom in stratified ‘personalised’ medicines and other innovative biotech technologies?

- Will current uncertainty in deciding the level of risk associated with the PPRS versus the statutory scheme put companies off from entering the United Kingdom?

- Will there be an initial UK NHS list price followed by a secret (commercial and in confidence) value-based price once NICE come to review technologies?

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Will any future legislation on innovative medicines include discussion on pricing, such as further exemptions to price cuts to encourage innovation?

References

2014 PPRS

The Health Service Medicines (Control of Prices and Supply of Information) (Amendment) Regulations 2013:
http://www.legislation.gov.uk/uksi/2013/2881/made

Statutory scheme for pricing branded medicines: Impact Assessment

June 2013 Statutory Regulations consultation – response:

Joint DH/ABPI statement on arrangements for pricing branded medicines from 2014
EU institutions have been promoting increased disclosure of pre-clinical and clinical trial data for some time, and the European Medicines Agency (‘the EMA’) seems determined to release as much data as possible after a marketing authorisation has been granted, considering there to be no legitimate impact on the companies concerned. In particular, since 2010 the significant change has been that the EMA has stated that it does not consider data from clinical trials supporting marketing authorisations to be commercially confidential.

The authorities claim to be responding to widespread public pressure for greater transparency, and, at times, it has seemed that there is little companies can do to stem the tide. Industry and intellectual property associations are objecting to the EMA’s increasingly unqualified policy in favour of disclosure, whereas researchers and national competent authorities have supported the EMA’s position. However, in pending litigation, the European Court will finally have an opportunity to review the legality of the new approach being adopted by the EMA.

Against this background, this comment sets out a summary of the current position in relation to disclosure of clinical trial data, and highlights some of the areas of development for the future.

The Pro-active Disclosure of Clinical Trial Data

The authorities release a large amount of data concerning clinical trials used to obtain marketing authorisations once the authorisation has been granted (or refused or withdrawn). This is to satisfy the obligations of transparency both in relation to human research and to the decisions of the regulatory agencies. For example, on authorisation of a medicinal product, the EMA (or the national equivalent for products subject to national approval and assessment through the decentralised and mutual recognition procedures) publishes a detailed European Public Assessment Report (‘EPAR’), which includes a summary of, and the conclusions reached on, the documentation submitted by the applicant.

Information, including results-based information, is also published on clinical trial registries. Member States have an obligation under section 11 of the Clinical Trials Directive 2001/20/EC to enter certain information about trials conducted in their territory onto the EudraCT database, and are required to make some of that information public. Commission guidance from October 2012 sets out the information to be made public, and states that, for all trials, result-related information should be supplied and made public after the completion of the trial, and not only after the grant of the marketing authorisation. The publication of clinical trial data is becoming an increasingly ‘hot topic’ with the EMA and national competent authorities, and while historically not many results-based data are included on the registries, it is likely that authorities will increasingly rely on this guidance to ‘force’ disclosure. In addition, due to the increased interest in clinical trial data, many companies have signed up to voluntary databases to increase the information that is made available, or have pledged to release data they hold.

However, the Commission guidance has been met with significant opposition from some companies, who consider the results of clinical trials to be confidential at least until the grant of the corresponding marketing authorisation. The lack of a common approach by industry has led some to believe...
that companies are hiding data and cannot be trusted to release data about their products. As a result, there is a large amount of negative press relating to clinical trial transparency, particular surrounding the new initiatives being proposed (discussed below). Much of this fails to recognise the complexity of the issues and the legitimate concerns that companies have, including in relation to protection of intellectual property in respect of which many commentators, perhaps understandably, fail to appreciate the implications of basic patent law.

Recent Developments Relating to Pro-active Disclosure

The EMA is committed to the pro-active publication of data supporting marketing authorisations, and, on 24 June 2013, published a draft policy on pro-active access to clinical trial data. The draft policy divides data into three categories:

- **Category 1:** documents containing confidential information which will not be disclosed. However, the EMA states that this will only be a small number of documents ‘in duly justified cases’, and the EMA does not, in principle, consider clinical trial data to be commercially confidential;
- **Category 2:** documents not containing confidential information or personal data which will be made available on the EMA’s website at the same time as publication of the EPAR;
- **Category 3:** patient-level data containing personal information, for which controlled access is necessary. In such cases, access will only be granted where appropriate assurances are in place, and a data-sharing agreement has been signed.

The consultation on the draft ended on 30 September 2013, and the EMA had intended the policy to come into force on 1 January 2014. However, due to the large number of responses received, the EMA has said that ‘the policy on publication of and access to clinical-trial data and an implementation plan will be discussed at the March 2014 Management Board meeting’. It is, therefore, unclear when the policy will be implemented and in what form. In addition, it is as yet unclear how this policy would apply for products authorised through the decentralised or mutual recognition procedures, and how it would be implemented by national competent authorities.

The discussions around the policy highlight, in particular, the significant differences of opinion on whether any data within the marketing authorisation dossier, other than manufacturing information, should be kept confidential. However, the EMA is committed to the policy, and sees greater openness as being in the public interest. Comments from industry have largely been met with suspicion, and there appears to be a lack of appreciation of how disclosure can affect the commercial interests of companies, particularly outside the EU.

Secondly, in July 2012, the European Commission published a Proposal for a new Clinical Trials Regulation, which includes the creation of a revised EU-wide database to contain details of clinical trials and which will be accessible to the public. After intense debate, in December 2013, the European Parliament and Council agreed on amendments to the draft. The agreed text states that the sponsor of a clinical trial conducted in the EU should submit a summary of the results of the clinical trial to the EU database within a year of the end of the trial. In addition, where the trial was intended to be used for obtaining a marketing authorisation, the applicant should submit the clinical study report 30 days after the marketing authorisation has been granted (or refused or withdrawn). However, although the recitals state that ‘... in general the data included in clinical study reports should not be considered commercially confidential once a marketing authorisation has been granted ...’, the (draft) Regulation does acknowledge that the EU database shall be publically accessible unless confidentiality is justified on the grounds of ‘protecting commercially confidential information, in particular through taking into account the status of the marketing authorisation for the medicinal product unless there is an overriding public interest in disclosure’. The Regulation, however, provides no definition of commercially confidential information. The next stage in the process is the reading of the proposal in the full European Parliament; this is currently scheduled for April 2014, and the fact of the recent agreement means that the proposal is likely to be adopted at this time.
The European Federation of Pharmaceutical Industries and Associations (EFPIA) and the US Pharmaceutical Research and Manufacturers of America (PhRMA) have responded to the public’s concerns by committing to increase the amount of information available to researchers, patients, and the public. In January, PhRMA and EFPIA’s joint ‘Principles for Responsible Clinical Trial Data Sharing’ came into operation.5 Under these principles, pharmaceutical companies will release, on request, anonymised patient- and study-level clinical trial data, along with full clinical study reports and protocols for US- and EU-approved medicines to ‘qualified scientific and medical researchers’. Such release is subject to contractual prohibitions, patient privacy protections, and confidential commercial information protections. Data requestors are required to provide a rationale for their proposed research, along with their analysis, publication and posting plans; any potential conflicts of interest, including potential competitive use of the data and the source of any research funding. Companies should establish scientific boards to review requests, and should commit to making the data request process transparent by publicly posting their review processes and the identity of external board members, including their existing relationships with the company.

**Responding to Requests under Freedom of Information Legislation**

The Treaty establishing the European Community notes that the European Union institutions should conduct their work as openly as possible, and that any citizen of the Union, and any natural or legal person residing or having its registered office in a Member State, should have a right of access to documents of the Union’s institutions. To implement this, Regulation 1049/2001/EC on access to documents grants EU citizens and legal entities an express right to access documents held by European institutions (this Regulation is not concerned only with bodies involved in pharmaceutical matters). Article 4 Regulation 1049/2001/EC contains exemptions to the right of disclosure, in particular where disclosure would undermine the protection of the commercial interests of a natural or legal person, including intellectual property, unless there is an overriding public interest in disclosure.

This general right of access to documents applies to documents held by the EMA, and the EMA has published a number of policy and guidance documents on access to documents (many Member States have equivalent legislation in their particular country, although the operation is different across the EU). Under previous versions of the policy, the EMA treated clinical trial data as confidential, and refused disclosure of such data. However, in 2010, the European Ombudsman, who investigates complaints of maladministration by EU institutions, delivered two decisions critical of the approach of the EMA. As a result, on 30 November 2010, the EMA published a new policy defining how it would respond to requests for access to documents.6 The EMA also published guidance on the application of Regulation 1049/2001/EC and the new policy to particular categories of documents and types of information held by the EMA. In relation to the documents within an application for a marketing authorisation (although the guidance does not specifically refer to clinical trial data), it is said that such documents will be considered as confidential prior to the final decision (approval, refusal, or withdrawal). However, once the relevant decision has been made, the documents will be considered to be public, and will be disclosed (subject to appropriate redactions).

**Court Proceedings in the EU**

The implementation of the policy by the EMA has been controversial. In particular, the EMA has said it does not consider data from clinical trials (or pre-clinical studies) to be commercially confidential, and that it has released a substantial number of documents since the operation of the policy. However, many companies consider documentation within the authorisation dossier to be confidential, and that disclosure would undermine the protection of their commercial interests (including intellectual property). As a result, the EMA is currently subject to two challenges to its policy.7 In both cases, the companies applied to the European General Court for annulment of the EMA’s decisions to disclose all the research-related documents contained in the marketing authorisation applications, and applied for interim orders preventing the EMA from disclosing that information pending the court’s decision. Several EU

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7) Cases T-44/13 and T-29/13 AbbVie v EMA, and Case T-73/13, InterMune UK and Others v EMA.
and US pharmaceutical trade and intellectual property rights associations have sought to intervene in relation to the substantive aspects of that litigation to support the applicants.

On 25 April 2013, the President of the General Court granted the applications for interim relief, with the result that the EMA may not disclose the contested parts of the requested documents pending the hearing of the substantive applications. The EMA expressed disappointment at the decision and, in July 2013, appealed to the Court of Justice. On appeal, on 28 November 2013, the Vice President of the Court of Justice found that the President of the General Court had made an error in law in his approach to determining whether the test for grant of interim measures was met. He therefore referred the case back to the President of the General Court for him to make a further assessment of the arguments and evidence. The application for interim measures, therefore, remains outstanding and the timeline for a final decision by the President of the General Court on whether interim relief should be granted is uncertain. The hearing in the substantive case is not expected to take place before the end of 2014.

As a result of the imminent legal analysis of its new policy as part of pending proceedings, the EMA appears to have adjusted its unqualified approach that pre-clinical and clinical data are never commercially confidential by arguing that, to the extent that the data can be treated as confidential (the disclosure of which might cause the company in question some commercial damage), the public interest in disclosure overrides the commercial interest in confidentiality.

Discussion

Since 2010, the EMA has taken a position of greater disclosure, and considers that all data should be disclosed after a marketing authorisation has been granted. The issue has become one that engages all stakeholders and has elicited strong debate, partly conditioned by the view that, in the past, industry demonstrated (it is said) that it could not be trusted to disclose adverse results of clinical trials or to present results in an unbiased fashion. In addition, the lack of common approach by the industry has led the EMA and others to question the concerns raised by some companies, particularly when there has been limited challenge to the increased disclosure until recently.

The debate is likely to be influenced by the outcome of the cases before the European Court which, while not being concerned with pro-active disclosure, raise the common underlying issues of whether companies can be harmed by disclosure and how far the public interest really requires disclosure. The cases should also clarify what can be considered to be confidential information within a marketing authorisation dossier, and what can be disclosed. This clarification will also be relevant to the interpretation of the new Clinical Trials Regulation, which does not define what is commercially confidential within the marketing authorisation dossier.

Given the EMA’s current stance, if a company is concerned that documents provided to the EMA contain commercially confidential information, it is advisable to tell the EMA that this is the case, and request to be notified if any request for access is received. The EMA has previously commented that consultation with the owner of the document is not always required, although recent practice suggests that the EMA is now notifying companies if requests are made for their documents. If a company is concerned about disclosure, it is important to explain why disclosure could damage the commercial interests of the company. We would also advise companies to participate in and respond to the various consultations and proposals that are being discussed in the EU, whether through the relevant industry body or directly, in order to highlight concerns they may have about disclosure.