

Challenges and Priorities for the Next Five Years

Response from the Genetic Interest Group

The Genetic Interest Group (GIG) is the UK alliance of support groups for individuals and families affected by all forms of genetic disorders, with approximately 140 organisations in membership. GIG's membership encompasses the spectrum of disease for rare single gene disorder to common complex conditions arising from the interactions between genes, and between genetic and environmental and lifestyle factors.

We welcome the opportunity to respond to this consultation and particularly endorse the proposed move to a more risk focused approach to regulations.

In formulating this response we concentrate on the questions in the consultation which are of particular relevance to our members and the patients and families they represent, recognising that others will be better qualified to comment on other aspects of this consultation.

Section 2: Safeguarding Public Health

Question 2. How can the Agency encourage you to report problems you experience (as a professional or a patient) with medicines and medical devices?

Under present arrangements the reporting of problems is separated from the provision of medicines, requiring a conscious effort on the part of the patients to report issues or problems that may arise as a result of taking a medicine. We would suggest that a feedback sheet incorporating the opportunity to comment positively as well as negatively be incorporated in medicine packs. This could ask questions about which aspects of a patient's disease were particularly well treated by medicine as well as those instances where problems arise. A freepost address would encourage the return of completed forms, thereby creating the opportunity to generate substantial post marketing information about the effectiveness of medicines. As an independent body the MHRA could aggregate data about given medicines and feed it back to manufacturers and others, improving the quality of pharmaceutical R&D without compromising patient confidentiality. A similar system could be developed for medical devices.

Question 3. What are your views on whether the Agency strikes the right balance on benefits, risks and informed choice in the actions it takes to safeguard health?

MHRA (and other EU and National Competent Authorities) should engage pro-actively with relevant patient groups (and groups of patients) establish acceptable levels of risk and benefit associated with particular diseases and given interventions. The resulting framework should be published and used to set the context within which prescribing decisions can be made. Assessment of risk and benefits varies between individuals and also at different stages in the life of an individual. What may be an acceptable level of risk for one individual may not be for another. Equally something acceptable at one point may be rejected by the patient at another time in their life. A high risk intervention that allows a person of 35 to spend a few more months with their children might be rejected in favour of palliation and a dignified death by the same individual at the age of 70.

Section 3: Information and Communication

Question 4. How should we best communicate the benefit-risk balance associated with medicines and medicinal devices?

Communication of risk is notoriously difficult. Some react well to ratios or percentages, whilst others need metaphors to grasp the concept. Care needs to be taken to assure that there is a shared understanding of what qualitative terms used to describe risk are meant to mean. How rare is a 'rare event'? What is a 'serious' adverse event? etc. Clinical geneticists and genetic counsellors working in the NHS have substantial expertise in risk communication and we recommend that this is captured and used as a basis for a framework for risk communication about medicines and medical devices. Any framework should be field tested with patient and consumer organisations to ensure that it is valid and reliable.

Question 7. What practical approaches could the Agency take to involve patients and the public – and reflect their views – in regulatory decision making?

The EMEA has patient members sitting on many of its committees. Patient representatives are full members with the same duties and responsibilities as any others and we would recommend MHRA adopts a similar approach. EMEA has also systematically promoted transparency through regular communication, the development of its web-site and consumer representation, again something which MHRA could develop at little or no additional cost. We believe that the inclusion of patient and consumer members of committees etc at EMEA has improved the quality of that organisation's outputs and would commend this approach to MHRA.

Section 4: Supporting Research and Innovation

Question 9. What are your views on the technological advances to which the Agency will need to respond in the coming years?

We believe it is likely that many of the technological advances in medicines that are likely to arise in the medium term will be subject to the Centralised Procedure and go through EMEA. MHRA will have a major role in contributing to European regulatory

decisions about tissue, cell and genetic therapies and will need to invest in creating expertise and capacity to respond to novel therapies – especially where those are for small patient populations where expertise in the specific disorder may be scarce. The strong research base in the UK provides a valuable resource for creating this capacity. This is not the case in many other Member States. This is likely to create a particular responsibility for the MHRA in creating an appropriate and proportionate framework for evaluating innovative therapies and the benefits they bring for patients. We recommend that exploratory discussions are held with other stakeholders in industry, academia and patient groups to generate models for achieving a satisfactory regulatory framework that is fit for purpose.

Question 10. What are your views on how the balance is currently struck between supporting research and innovation, and taking a precautionary approach to prevent harm?

In our view the pendulum has moved too far in the direction of avoiding harm at the expense of research and innovation. A more open dialogue with patients and families would help redress this balance and promote informed decisions about risks and benefits by those to whom medicines are prescribed.

Question 11. Do you have a view on the proposal to move to earlier, conditional licensing of medicines?

We endorse the movement to earlier conditional licensing and post-marketing surveillance, especially for therapies for rare conditions where traditional phase 3 trials may not be feasible, and for innovative interventions where the experience of real life application of such therapies is likely to improve understanding of risks and benefits more quickly than insisting on traditional licensing frameworks. We suggest that the flexibility of the system for compassionate use developed in France could be adopted in the UK.

Section 5: The European and International Landscape

Question 12. What are your views on how the Agency should balance its national and European activities?

Patients throughout the EU want medicines that meet agreed standards for safety and efficacy. Many of the therapies that may benefit the families in GIG's member groups will be subject to the centralised procedure, and we suggest it would be appropriate for national competent authorities to develop complementary areas of expertise rather than seeking to be all things to all people. This would improve the quality of decision making and help secure a common European standard that would benefit patients, clinicians and industry alike.

Question 13. Do you have views on the future development of the EU framework for medicines and/or medical devices regulation? Are there changes or improvements you would want the UK to support?

We would like to see regulations for devices and diagnostics that address clinical utility as well as technical effectiveness. We should also like to see a public information

campaign informing patients and citizens of the differences between regulations for the approval of medicines and for the approval of devices and diagnostics as these are not widely appreciated. We would also like to see more vigorous application of existing consumer protection legislation in respect of the information provided to people contemplating the purchase of an over the counter test. We recognise that this is outside the MHRA's remit at present, but would suggest that the Agency could act as a technical resource to support other bodies such as Trading Standards.

Question 14. Where should the MHRA target its international cooperation work (either in terms of countries, or areas of work)?

Drugs and devices for genetic diseases, and gene derived therapies produced by the biotech, industry are likely to be areas where regulation with robust expertise are in short supply globally, and we suggest that, by developing expertise and capacity in these areas MHRA would be able to provide a services to the global patient community that would be greatly valued by those who stand to benefit from prompt, effective, proportionate regulation.

Question 16. Do you have proposals for further Better Regulation improvements in the regulation of medicines and medical devices?

We value the intention to reduce the regulatory burden where possible, and would encourage the MHRA to engage in early consultation with manufacturers and also with NICE and other similar bodies in the UK and the EU to agree a common data set for the evaluation of quality, safety and efficacy and also of clinical and cost effectiveness. This would encourage innovation and promote the development of products for unmet medical needs rather than drawing manufacturers into safer areas such as "me-too" and lifestyle drugs because the nature of the evidence base needed to gain approval would be clear and understood by all concerned from an earlier stage in the development of drugs and devices.

We would be happy to add the above comments either face to face or in writing it this would be helpful.

A handwritten signature in black ink that reads "Alastair Kent". The signature is written in a cursive, slightly slanted style.

Alastair Kent
Director
29th October 2007