The Academy of Medical Sciences

FORUM Annual Lecture 2010: Dr Margaret Hamburg

This is a summary of the Academy of Medical Sciences' 2010 FORUM Annual Lecture, **'The importance of regulatory science to the healthcare portfolio'** delivered by **Dr Margaret Hamburg, Commissioner, US Food and Drug Administration**, on 13 October 2010. A video of the lecture is available from http://www.acmedsci.ac.uk/index.php?pid=44&evid=156.

Summary

Dr Hamburg reported that as well as being a regulatory body, the US Food and Drug Administration (FDA) is an essential public health agency, which aims to promote and protect the health of people. However, achieving this objective is hampered because we have not adequately delivered on the opportunities offered by the tremendous breakthroughs being made in science and technology. There is a gap between scientific advances and improved patient care because we do not currently translate new findings effectively into the real-world applications and products that improve patient care; such applications could enable us to better diagnose, treat and ultimately prevent and cure diseases. Further, the nature of research and development (R&D) itself is changing as the number of new therapies emerging falls, but the costs rise.

To improve capability in this area, Dr Hamburg believes that regulators must streamline and modernise regulatory pathways, and efforts to do so must include international collaboration, information sharing and greater standardisation. To support this objective, regulatory science should be strengthened: the science and tools required to help develop, assess, and evaluate products for their safety, efficacy, quality and performance. New approaches should be sought to accelerate and improve the development, review, approval and ongoing oversight of medical products. Promoting innovations in regulatory science, through raising its profile and greater investment in the discipline, will help to yield the benefits that should be emerging from the rapid progress seen in biomedical science, for example, enabling:

- Earlier elimination of ineffective products during their development – not after authorisation or at the end of a costly development programme.
- More effective identification of new drug targets.
- Innovative clinical trial design, new analytic approaches and application of advances in IT/data mining.
- Faster delivery of products to patients.

The FDA is working collaboratively to advance the field of regulatory science and is hoping to expand its activities, for example by establishing centres of excellence in regulatory science. These would most likely sit in the academic sector, but would bring scientists together from across the sectors. The impacts of regulatory science are potentially wide-ranging, it is an essential part of the overall scientific enterprise and an important driver of health and prosperity. A number of specific examples illustrate some of the critical areas where advances in regulatory science can help to improve health and healthcare:

Realising the era of personalised medicine

Work is ongoing to identify, characterise, and qualify biomarkers for regulatory and clinical use, the applications of which include:

- Enhanced preclinical toxicity studies, which could lead to earlier identification of ineffective or unsafe drug candidates. For example, newly identified biomarkers are being used to indicate drug-induced kidney toxicity in preclinical animal models that were co-validated and simultaneous accepted by the FDA and EMA.
- Optimisation of drug delivery and dosing so that patients receive the greatest benefits with the lowest risk. For example, genetic tests that improve estimates for a patient's optimal warfarin dose.
- In cancer, regulatory science is working to identify potential tumour markers that could indicate whether a cancer will respond to a specific or combination therapy.

Rethinking the clinical research methods used to test emerging therapeutic approaches

Regulatory science is exploring how to design clinical research methods that test emerging therapeutics more effectively. New trial methodologies should be developed and implemented that maintain scientific rigour and protection from biases, but also provide for more rapid answers, more targeted answers, and smaller study populations than may be possible with traditional large population based approaches. This includes adaptive trial designs and more advanced analytical techniques.

Preparing for an increasingly broad range of diseases

The world faces major health challenges in the future, demanding that we prepare for a broad range of diseases, from infectious diseases such as SARS and pandemic influenza, to new and unexpected events including the threat of biological, chemical or radiological terrorist attacks. Such events will emerge in uncertain contexts with limited opportunities for human clinical testing and few well established animal models. Regulatory science can help to develop appropriate tools and regulatory pathways that can facilitate the rapid testing and evaluation of diagnostics, drugs and vaccines for these threats. For example, new regulatory tools could create greater flexibility in product development and manufacturing, while new statistical methods could help to model efficacy with limited data to help address outstanding scientific issues.

Keeping up with emerging technologies

Nanotechnology and stem cells offer great potential to improving medical science techniques, but we must also understand the associated safety risks. For example, applications in nanotechnology could facilitate more targeted delivery of drugs to specific cells, but may also contribute to tissue inflammation and damage or create specific risks yet unseen with non-nano-sized particles. Work in regulatory science can develop methods for determining how to assess the near- and long-term safety concerns of such emerging technologies.

Enhancing safety science

Regulators must monitor products over their entire lifetime on the market – from first introduction into humans throughout its presence on the general market. Central to this endeavour is the science of pharmacovigilance, where techniques are used to monitor the post-market environment for safety signals and other risk concerns and to test hypotheses of ongoing concerns. Work in regulatory science can enable more rapid and meaningful evaluation of emerging concerns and ultimately ensure that risks and benefits are balanced appropriately for patients.