# INNOVATIVE HEALTH TECHNOLOGIES PROGRAMME

# Regulation of Innovative Pharmaceuticals in the EU and the US

## KEY FINDINGS

What are the central social scientific features of the regulation of innovative pharmaceuticals in the US and (supranational) EU, and how do these two systems compare? What are the citizenship dynamics involved in these regulatory systems, and how do they relate to innovation, therapeutic advance and public health? What are the relationships between the regulation of innovative pharmaceuticals and various interested parties: the pharmaceutical industry; the executive arm of government; the legislature; and the interests of patients and public health? What is the nature of the regulatory science regarding innovative pharmaceuticals in the two systems? What are the explanations for inconsistencies in regulation of some case-study innovative pharmaceuticals between the two territories?

## RESEARCH FINDINGS

- In both territories post-1995 various regulatory mechanisms have been established to accelerate the review and marketing approval of innovative pharmaceuticals.
- Regulators, industry and government bodies frequently justify this regulatory approach on the grounds that it gives patients faster access to 'innovative' drugs that they need.
- In fact, the main, consistent and relentless driving force for the acceleration of drug review and marketing approval in the US of drugs, whether they offer significant therapeutic advance to patients or not, has come from the pharmaceutical industry and those ideologically committed to a 'deregulatory' political agenda in various parts of the EU and US governments.
- These accelerated approval mechanisms for 'innovative' pharmaceuticals are not necessarily meeting desperate patient needs and may raise many problems for doctors and patients.
- Many aspects of these regulatory reforms are a shift away from citizenship rights to security in health in the form of secure evidence that drugs to which patients (including those who are desperately ill) are exposed are efficacious and safe and towards citizenship rights to private property in the form of pharmaceutical companies having the right to market their drugs with less evidence of efficacy and safety.


**RESEARCH TEAM**

John Abraham, Professor of Sociology & Co-director of the Centre for Research in Health and Medicine (CRHaM), University of Sussex

Dr Courtney Davis, Research Fellow in Sociology and CRHaM, University of Sussex



In collaboration with and co-sponsored by MRC Medical Research Council

Within the regulatory systems of the EU and the US, the definition of an 'innovative' pharmaceutical does not necessarily mean that it offers significant therapeutic advance. Nevertheless, in both territories post-1995 various regulatory mechanisms have been established to accelerate the review and marketing approval of innovative pharmaceuticals. This has resulted in fewer regulatory checks and/or less evidence regarding the efficacy and safety of new drugs passing through these mechanisms. Regulators, industry and government bodies frequently justify this regulatory approach on the grounds that it gives patients faster access to 'innovative' drugs that they need. Indeed, they and some academics have argued that such regulatory mechanisms have been established in response to patient demand. However, findings from this research demonstrate that the main, consistent and relentless driving force for the acceleration of drug review and marketing approval in the US of drugs, whether they offer significant therapeutic advance to patients or not, has come from the pharmaceutical industry and those ideologically committed to a deregulatory political agenda in Congress and various Administrations. Similarly, in the EU the centralised regulations for innovative pharmaceuticals, including various arrangements for exceptional acceleration, resulted mainly from demands of the industry and the Commission, rather than patient groups.

The fact that the pharmaceutical industry and various arms of government in the EU and the US have driven the acceleration of drug review and marketing approval, rather than patients groups does not necessarily mean that it is impossible for those reforms to be in patients' interests; it demonstrates only that the reforms are neither primarily a response to, nor primarily designed to meet, patients' interests. While these reforms are certainly in the (short-term) interests of industry because companies can market their 'innovative' pharmaceuticals more quickly and easily, it is possible, in theory and in practice, that they are also coincidentally in the interests of patients.

This 'coincidence of interests' hypothesis was investigated primarily by analysis of the consequences of the accelerated review and marketing approval on the regulatory science and decision-making regarding 10 'innovative' pharmaceuticals developed to treat serious or life-threatening conditions. While it would be foolish to claim that no patients have ever benefited from these accelerated approval mechanisms, evidence from these case studies about how they work in practice does not support the idea that, even though industry and deregulatory political agendas have been the main driving force behind these regulatory reforms, the reforms are nevertheless in patients' interests by 'coincidence'. Rather, these accelerated approval mechanisms for 'innovative' pharmaceuticals are not necessarily meeting desperate patient needs and may raise many problems for doctors and patients. Findings from the case studies also suggest that the EU centralised procedure may be more a precautionary regulatory agency (about exposing patients

to drugs with problematic safety and efficacy profiles) than the one that the FDA has become since 1995. This may be because, the FDA has a much more hierarchical structure compared with the more collective approach of the CPMP, within which CPMP members are employed by their national regulatory agencies, rather than the supranational regulatory institutions, thus limiting the responsiveness of CPMP evaluations about individual drugs to the organisational goals of accelerated marketing approvals at the behest of industry demands.

Many aspects of these regulatory reforms are a shift away from citizenship rights to security in health in the form of secure evidence that drugs to which patients (including those who are desperately ill) are exposed are efficacious and safe and towards citizenship rights to private property in the form of pharmaceutical companies having the right to market their drugs with less evidence of efficacy and safety. This shift in citizenship rights has been more dramatic and substantial in the US over the last 10-15 years. As occurred earlier with EU drug regulation, the FDA has become substantially dependent on industry fees; has come to view pharmaceutical companies as its customers; has strict targets for speed of drug review (whether 'innovative' drugs or not) as demanded by industry; and had abandoned any pre-existing arms-length or adversarial workings with industry in favour of flexible arrangements involving regular meetings with industry. In terms of organisational relations, the FDA has converged with the EU model and in some respects overtaken its enthusiasm for regulation by partnership with industry and its goals.

It is recommended that if drug regulatory agencies in the EU and US wish to prioritise the interests of patients and public health, then they should fundamentally reconsider the enormous emphasis currently given to accelerating drug review and marketing approval, especially as the therapeutic advance presented by many pharmaceuticals defined as 'innovative' is not particularly significant. Regulatory agencies should require adequate evidence of efficacy and safety data for 'innovative' pharmaceuticals before marketing approval, including evidence regarding appropriate clinical outcomes and therapeutic advance where possible. Individual patients should not be denied drugs that give them benefit during trials, but these drugs should be made available to those patients on a compassionate basis, if it is found that there is insufficient evidence to approve the drug more widely. This is vastly preferable to reforms that have altered the adequacy of time and evidence upon which 'innovative' pharmaceuticals are approved in general.

# About the Project

The research is concerned with the regulation of prescription medical drugs, which may be regarded as 'innovative' pharmaceuticals, in the US and the supranational EU This is the first piece of social science to investigate and compare the EU's supranational centralised regulation of innovative pharmaceuticals with drug regulation in the US by the FDA. The focus is mainly on the period from 1995-2003 because 1995 is when the EU's supranational centralised procedure began. It is not directly concerned cost-effectiveness as these are outside the regulatory responsibilities of the US FDA and the EU centralised procedure.

The research involved investigation of the two regulatory systems in general plus their approach to a number of case-study 'innovative' pharmaceuticals, in particular. We reviewed numerous regulatory documents from the EMEA, FDA, US Congress, EU Parliament, US Government Administrations and the European Commission. Documents by the US and European pharmaceutical industry associations were examined as were documents from individual drug companies and various patient and public health advocacy organisations in the EU and US. Regarding the case-study drugs, systematic scientific and medical literature reviews were conducted in relation to the regulatory science gathered from the agencies and other sources.

These documentary sources were complemented by interviews in the EU and the US with current and former regulators; current and former pharmaceutical industry scientists or representatives, and other interested parties, such as those in the legislature, clinical investigators, public health advocacy organisations and patients groups. As envisaged in the proposal, a total of 109 interviews were sought. The documentary and interview data were analysed in accordance with the following objectives:
to examine and compare the regulation of innovative pharmaceuticals in the (supranational) EU and the US; to explore the citizenship dynamics involved in these regulatory systems; to examine the relationships between the regulation of innovative pharmaceuticals and various interested parties, such as the pharmaceutical industry, the executive arm of government, the legislature, and the interests of patients and public health; to investigate the regulatory science of, and explain inconsistencies in regulation of, some case-study innovative pharmaceuticals between the two territories.

**For further information contact:**
**Professor Andrew Webster, IHT Programme Director**
**Department of Sociology, University of York, Heslington, York YO10 5DD**
**Tel: +44 1904 43 3064/4740 ◆ Fax: +44 1904 43 4702/3043 ◆ E-mail: iht@york.ac.uk**
**Web site: http://www.york.ac.uk/res/iht/**