

**REPORT OF RISING POWERS POLICY WORKSHOP:
MODELS OF BIOMEDICAL INNOVATION AND CONSUMER DEMAND**

Held on 29 May 2014 at King's College London

This report has been compiled by Saheli Datta
and approved by the workshop participants

A policy workshop was held on 29 May 2014 at King's College London as part of the ESRC funded research 'State strategies of governance in biomedical innovation: the impact of China and India'. This project explores emerging innovation dynamics and trans-national governance in the context of the increasing importance of the life sciences and technologies in countries' and regions' competitive and collaborative economic strategies, and the recognition that biomedical sciences raise difficult questions of ethics and of social impact. The project involves a series of workshops, whose results are designed to inform public policy-making in a range of fields in regenerative and personalized medicine in the UK and the 'Rising Powers' of India and China. This workshop was the fourth in the series (Beijing, 2013 and New Delhi, 2014) and the second to be held in London since 15 March 2013.

The workshop was chaired by the research Principal Investigator Brian Salter, Professor of Politics in the Department of Political Economy, King's College London and supported by Research Associate Dr Yinhua Zhou and PhD Researcher and Coordinator Ms Saheli Datta. Attendance was by invitation only. The invitees, sixteen in total, ranging from clinicians, academics, researchers, lawyers, bioethicists and regulators, represented a wide range of public and private sector organizations including National Institute of Health and Care Excellence (NICE), Cell Therapy Catapult (CTC), Intercytex, Kinetics Corporation Incorporated, Association of British Healthcare Industries, DeBRA International (patient organization), Lawford Davies and Denoon, University Hospital of South Manchester, University of Exeter, University of Manchester, Cardiff Business School, King's College London and Spanish Research Council.

The objective of the workshop was to better understand the extent to which new models of innovation responsive to consumer demand may be constructed within the existing regulatory framework? Traditionally, the model of innovation employed in biomedicine has been driven by the preferences and concerns of the supply-side – principally science, medicine and industry. However, the advent of a global market in healthcare where consumers make their own choices regarding the treatment they want, when, where and how, has energized a demand side view of what the innovation process should look like. Cell therapy is not immune from these developments and new models of innovation have emerged, particularly in the emerging economies, which deliver new treatments on a much shorter timescale than that offered by the traditional model. As a result, there is now a vibrant global market in cell therapies fuelled by a supply side employing non-traditional models of innovation.

The exclusion of North America, Europe and Japan from much of this new market because of their adherence to the traditional innovation model raises important political, regulatory and ethical issues that this workshop aimed to address with the objective of moving the debate forward in a constructive and practical manner, focusing in particular on the UK's

position. The workshop opened with an analysis of “Health consumers, markets and cell therapy innovation: the global context” by Professor Brian Salter ([presentation attached](#)). The presentation looked at the political forces shaping innovation models in the global market of cell therapies, focusing on the role of consumers in this market and how it shapes the demand-supply relationship and the nature of the market’s engagement with different models of stem cell therapy innovation (see Salter, Zhou and Datta, 2014a; 2014b).

The second session subjected this analysis to ethical scrutiny, drawing out the value assumptions that underlie competing innovation models and identifying the ethical implications of a shift towards a more consumer oriented approach. Professor Christine Hauskeller, Senior Lecturer at the University of Exeter, Exeter, UK, provided the bioethical perspective- the patient as a consumer and “the ethics of innovation: balancing protection and participation” ([presentation attached](#)).

Given this political and ethical context with its associated challenges and opportunities, the final session addressed the position of the UK in the cell therapy market and the ability of its regulatory structures to respond to the economic and political demands of the global competition for advantage. Dr Paul Kemp, Chief Executive and Chief Scientific Officer at Intercytex and involved in commercial regenerative medicine for over 25 years, provided the industry perspective “combining scientific and medical innovation into the progressive translation of cellular therapies” ([presentation attached](#)).

This report is organised as follows. We begin with an outline of the issues identified and questions raised by each presentation. This is followed by a brief report of the discussion from the perspective of the patient-as-a-consumer-of-health-services, the regulator, the bioethicist and the industry. Finally we conclude that introducing flexibilities in the current innovation model towards more patient-industry interaction and a more democratic patient-clinician relationship presents the best way forward to stem steadily declining translation rates from stem cell research to therapies. The workshop helps provide pointers for future research and policy development to understand the ‘global biopolitics’ of consumer-demand and policy implications of this innovative field.

ISSUES IDENTIFIED BY THE WORKSHOP

1) Political forces shaping innovation models in the global market of cell therapies.

Professor Salter’s introductory presentation “Health consumers, markets and cell therapy innovation: the global context” ([presentation attached](#)) sought to discuss key issues as below,

- What should be the role of the health consumer in cell therapy innovation?
- To what extent is the consumer demand for new therapies reflected in political demands for new or revised models of innovation?
- What is the contribution of practice based models of medical innovation to a more consumer friendly approach to innovation.
- What are the implications of consumer demand for the roles of scientists, clinicians and industrialists in cell therapy innovation?
- What regulatory changes would enhance the capacity of consumers to make informed choices and risk judgements about cell therapies?

2) Ethical implications of a shift towards a more consumer oriented approach.

Professor Christine Hauskeller, Senior Lecturer at the University of Exeter, Exeter, UK, a bioethicist, provided the perspective of the patient as a consumer- “the ethics of innovation: balancing protection and participation” ([presentation attached](#)). The presentation sought to discuss the issues below,

- The patient as consumer
- The needs and expectations of patients and the legitimacy of authoritative institutions – science, medicine, regulators
- Who decides the best way forward for cell therapy?
- Non-linear innovation. Successful medical therapies are dependent on their consumers, the patients, in different way than other innovations.
- The consumers ability to judge their own best interest
- Ethical issue relating to unequal access to resources
- Should individuals be free/encouraged to travel in order to get what they (and possibly their doctor) believe could benefit them?

3) UK’s competitiveness in the current regulatory environment.

Dr Paul Kemp, Chief Executive and Chief Scientific Officer at Intercytex and involved in commercial regenerative medicine for over 25 years, provided the industry perspective “combining scientific and medical innovation into the progressive translation of cellular therapies” ([presentation attached](#)). The presentation sought to discuss the issues below,

Issues

- How can existing legislation, UK, EU and ROW best be utilised to accelerate iterative development of effective therapies?
- The role of the practice of medicine in cell therapy. How can this be used to develop the best treatment protocol and how can this inform pivotal clinical studies?
- The role of the patient in the development process
- Progressive reimbursement. What sources are available? How would pricing be determined? How could risk sharing be used?
- How can the clinician/industrial interface be used in order to inform product development and application within the current legislative framework?
- Managing ethics, hype, conflicts of interest and social perceptions
- Threats and opportunities of medical tourism to the UK landscape
- What might the future look like if nothing is changed?

DISCUSSION

The perspective of the patient as a consumer

The patient perspective centered on the need to increase the responsiveness of the existing model of bioinnovation in general and stem cell therapy in particular to patient needs and demands. Patient organizations, representing the patient’s voice, felt that ‘much’ more needed to be done in terms of current healthcare innovation systems’ responsiveness to patient needs. The patients’ feeling about the existing innovation models responsiveness, as

expressed by the patient organisations and a few clinicians, is a pervading ‘paternalistic’ attitude that necessarily assumes patients as passive agents or ‘listeners’ with little agency either in understanding or making personal healthcare decisions who need to be told, by clinicians and regulators, what is best for them. Furthermore, patients view current regulatory environments as ‘rationing access’ of patients to innovative treatments and attach less importance to ‘quality’ and ‘regulatory controls,’ in comparison to regulators and bioethicists. The inherent emasculation of patients, as without agency, by the medical and scientific community is a view increasingly shared by a number of empirical studies of patient networks, patient-run blogs, chat rooms, social media etc (Chen and Gottweis, 2013; Elkin, 2008; Fox and Duggan, 2013; Morgan et.al, 2011). Furthermore, patients feel that current research programmes in UK unfairly privilege ‘intervention based research’ rather than ‘preventive treatments-based research’ and that this direction needs to change not only to save on costly interventions including hospitalization and care costs but also for a healthier Britain.

The regulatory perspective

From the regulatory perspective, there was an acknowledgement of the need, from patients and the industry, for ‘more’ systemic flexibilities in the existing innovation model both in terms of democratizing patient access as well as increasing UK’s competitiveness in the global bioeconomy. The recent introduction (in April 2014) of systemic flexibilities like the fast-tracking of the innovative pathway through Promising Innovative Medicine (PIM) and Early Access to Medicines Scheme (EAMS) along with existing regulatory arrangements like ‘specials,’ and ‘hospital exemptions,’ were cited as examples of regulatory efforts to address issues of patient access while making UK companies more competitive. The regulatory move, from stringently regulated case-by-case assessment approach of the existing ‘specials’ and ‘hospital exemption’ to a more generic and less case-by-case-based assessment approach of the recently launched PIM and EMAS, were cited as a reiteration of the regulatory apparatus’ willingness to increase responsiveness to increasingly elastic consumer demand for innovative treatments. In turn, the responsiveness of the previously inflexible regulatory apparatus, served to affirm not only the regulators acknowledgement of the pervading ‘paternalism’ of the existing model with regards to patient access but also the need for change.

On their part, regulators felt that ‘risk averse’ Research and Steering Committees¹, averse to being seen as the ‘one’ that approved a research that ‘went wrong,’ posed a significant challenge for change initiatives. In summary, the push for further systemic flexibilities appeared to be well underway, from the regulators view, with the UK Medicines and Healthcare Regulator Agency (MHRA) expressing keen interest in working with the EU to introduce changes that would further narrow the ‘valley of death’ while improving public access to innovative treatments like ‘experimental’ stem cell therapies.

The bioethical perspective

The bioethical view, hinged on the patient narrative, was presented in two divergent strands. One view was that the ethics of stem cell science as practiced in India and China, referring to patients being given unproven stem cell treatments and stem cell clinics “trading on hope” (Qiu, 2009) ran ‘parallel’ to that in the west. This view is well established in literature (Taylor

¹ Expert groups responsible for assessing suitability of innovative therapies (e.g. ethical, legal, social aspects, scientific merit etc) before progressing it to the next phase of the innovation process.

et.al, 2008; Murdoch and Scott, 2010; Pownall, 2010). This view necessarily assumes patients to be passive agents unable to make the ‘right’ personal healthcare choices and rejects notions of the patient as ‘consumers’ that is as socio-economic agents with political agency in an increasingly demand driven global ‘bioeconomy.’ The other view, questions the traditional power relations between the ‘passive patient’ subordinated to the ‘knowledge expert clinician,’ and considers the patient as a well-informed and an important socio-political and economic actor with agency to make rational personal healthcare decisions. The latter view is grounded in the fact that patients no longer suffer from information asymmetries commonly associated with the snake-oil healers and quacks of the 17th and 18th centuries. Rather, recent studies show that patients achieve unprecedented levels of knowledge gain from the internet, social media, chat rooms, blogs, online healthcare resources etc (Adams, 2011; Gottweis & Chen, 2014).

The industry perspective

The view from industry focused on current regulations restricting commercial entities’ access to patients and held as partly responsible for low translation rates from clinical trials to marketing licences. Two instances of regulatory restrictions were held as major roadblocks. First, the fact that industry (private sector developers of new drugs and therapies) is prevented from talking to patients involved in clinical trials prevents them from receiving key feedback about the intervention under trial and the trialling process. The view is that access to key patient feedback and related clinical trial data through a closely regulated interactive process can turnaround declining translation rates from research to therapeutic products. Second, the fact that industry is unable to ‘talk’ to clinicians to pursue commercialization efforts of a successful ‘experiment’ or ‘trial’² presents a roadblock to better translation rates. The view is that flexibilities in this area, provided it is stringently regulated and monitored to prevent abuse, could see greater commercialization successes. Furthermore, these flexibilities could make UK based small and medium enterprises, currently responsible for most of the downstream commercialization activities of stem cell research in the UK, more competitive globally. However, a profound and concomitant appreciation of the ‘ethical pot’ involved in deregulating clinician-industry linkages was voiced along with the need for stringent checks and balances to make deregulation ‘work’ for the benefit of the Regenerative Medicine (RM) industry. In this regard, it was felt that separating the different components of RM, like cell therapy, transplantation medicine, non-cell therapy based interventions like cancer care, medical devices, wound care etc, in popular discourse, policy documents, media would help to ‘free’ areas of RM that had little to do with human embryonic stem cells but are currently lumped together in the same ‘ethical meting pot’ surrounding ‘embryo research.’

The way forward

Introducing flexibilities in the current innovation model towards more patient-industry interaction and a more democratic patient-clinician relationship were considered the best way forward. Moving towards medical governance with ‘self governance is on its way out,’ e.g. using enforceable regulations of medical practitioners and clinicians, was suggested as a possible model of democratising the patient-clinician relationship while protecting both

² Under current regulations, commercial entities restricted from approaching clinicians to pursue commercialization efforts after a successful product trial. However, the reverse is allowed, that is clinicians are allowed to approach commercial entities for commercialization following trials.

patients and clinicians from malpractice and abuse. In this regard, the recently launched Japanese model allowing drugs or therapies proven 'safe' to be tested over a seven year period for 'efficacy' by clinicians in real world (i.e. non-clinical trial) settings, provided a possible alternative to the existing model. However, the fact that changes in Japanese regulations were industry driven rather than being driven by patient groups was considered thought provoking. It was felt that a model of innovation closely resembling the Japanese model, but using a licensing scheme for clinicians to allow for a 'gradual emergence of efficacy,' could be used in the UK to increase commercialization prospects of its extensive stem cell research capabilities.

In terms of pointers for future research, exploring the various aspects of transnational governance and policy development to understand the 'global biopolitics' of consumer-demand and policy implications of this innovative field is important. Notably, research into the intricacies of transnational governance in stem cell research, using the case study of stem cell research and therapies in China and India, is being conducted by Ms Saheli Datta for her doctoral research and as part of the ESRC Rising Powers research project.

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