



Cooksey Review on Health Research
Response by the BioIndustry Association
July 2006

EXECUTIVE SUMMARY

Introduction

The BioIndustry Association (BIA) welcomed the Chancellor of the Exchequer's Budget announcement in March this year of a single, ring-fenced budget to support health research. We congratulate the Government on conducting this important review, and welcome the opportunity to submit a response.

There is a significant opportunity for the single budget to support the vital biomedical R&D being conducted in this country to develop new medicines for patients.

The UK is a world leader in biomedical research, second only to the US and one of the few fields where the UK is a global leader with good prospects of remaining competitive for the foreseeable future, even in the face of growing competition from countries such as India and China.

Biological medicines have the potential to address unmet medical need. More than 250 million patients worldwide have already benefited from approved biotech medicines and therapies to treat or prevent heart attacks, multiple sclerosis, breast cancer, cystic fibrosis and leukaemia, for example.

Yet the rate of uptake of innovative medicines in the UK is notoriously low, which does not serve the best interests of patients in this country. Furthermore, just 10,000 of the 30,000 known diseases have treatments available. Improving the uptake of innovative medicines is key not only for the UK to remain an attractive research location, but also for UK patients to fully benefit from the results of this research.

Recommendations

The BIA believes that its recommendations in this submission would help to position the UK as a world leading location for innovative biomedical research.

The following recommendations are proposed:

- **Proof of Concept Scheme**

The BIA believes that there is an opportunity to explore the possibility of introducing, through the Medical Research fund, a scheme/schemes that would:

- a) mirror the Scottish Proof of Concept scheme, i.e. provide funding for high tech projects originating in academia; and
- b) also provide seed funding for early stage high tech companies, mirroring the two new investment funds announced earlier this year by Scottish Enterprise to complement the Proof of Concept scheme.

This would have the dual impact of improving the commercialisation of academic medical research and improving the financial environment for early stage bioscience companies, both of which would ultimately be of benefit to patients.

In this submission the BIA examines how the Scottish scheme operates, the successes it has achieved and the benefits it has delivered. In addition, the paper sets out evidence for introducing a complementary scheme to cover seed funding for early stage high tech companies, by taking as an example the funding gap for bioscience companies at that stage.

- **Capturing and Exploiting Intellectual Property (IP)**

There is a massive opportunity for the NHS in the form of the new single health research budget. An effective system for the protection and exploitation of IP arising from funded biomedical research will be essential to give the best chance of innovations and discoveries reaching patients and at the same time resulting in valuable knowledge transfer that can grow the UK biomedical sector. The IP strategy has to be appropriate for any individual innovation and the process of engaging with commercialisation partners has to be established. Clarity of IPR ownership is perhaps the most important issue.

The BIA recommends:

- Establishing clear IPR ownership and clear responsibility for IPR exploitation as prerequisites for release of a grant;
- Establishing clear value split between parties resulting from IPR exploitation as a prerequisite for release of a grant (or a clear process for determining a split);
- Considering centres of excellence for IPR exploitation using existing organisations where appropriate;
- Considering the creation of specialist TTOs where none exist;
- Allocating a proportion of the fund budget for patenting and IPR exploitation;
- If the appropriate TTO elects (in a timely fashion) not to patent or exploit a technology/innovation, the inventors should be free to exploit with a defined small percentage of reward returning to the fund/institution;
- The decision on what to patent and what to exploit needs to be rapid and effective so that inventors whose ideas are not backed can still be exploited and benefit patients and the economy;
- Considering a 'co-investment fund' to kick start and maintain equity positions in spin-outs;
- Establishing a mechanism for IPR pooling across disparate groups; and
- Establish a strategy for exploitation of the IPR embodied in NHS patient/treatment /outcome records.

- **Supporting Connecting for Health**

The drug development process is driven by the need for actionable and accurate deployment of new medicines. Real time clinical data is at the heart of improved discovery data and information to support the drug selection and development process. The BIA therefore believes that Connecting for Health could make a major contribution to the discovery and development of medicines. This is a potential "USP" for research and biomedical R&D investment in the UK.

Optimising clinical trial design, operation and data outcomes will play a major role in producing greater medical benefits to patients. Ensuring that the new system results in real benefits for UK patients, Connecting for Health will make the UK a leader in clinical research.

The BIA would therefore like to reiterate the huge importance of Connecting for Health in improving patient access to innovative medicines and to ensure Government support for it.

- **Reimbursing Medicinal Products for Compassionate Use**

The BIA supports the new EU policy with regard to supply and distribution of an unauthorised medicinal product for compassionate use. However, it falls short of recognising that products supplied for compassionate use are equivalent to authorised products for the purpose of reimbursement.

By way of contrast, a system of reimbursement for these products is in place in France – the Authorisation Temporaire d’Utilisation (ATU) de cohort system. French patients are already benefiting – there is no sound reason why UK patients should not. This could also help to improve the UK’s slow rate of uptake of innovative medicines.

The BIA recommends:

- A policy review so that innovative medicines that receive a favourable opinion from the EMEA Advisory Committee (CHMP), under the new EU provisions for compassionate use to a group of patients, are used in the NHS and are included within the scope of the national health insurance system as soon as the CHMP adopts its opinion. This will allow quick access of life saving drugs by the NHS to patients in need; and
- The introduction by the DH of a new scheme allowing the pricing and reimbursement from a central fund of innovative drugs made available for compassionate reasons to a group of patients.

- **Encouraging the Development of Medicines for Smaller Patient Cohorts**

There are some 8,000 rare diseases, many of which are life-threatening and/or seriously debilitating. For most of them, there is no access to effective medical treatments. Given that 70-80 per cent of these rare diseases have a genetic origin, bioscience is an important tool to develop treatments for them.

Due to the fact that there is high unmet medical need and due to the small population that each disease affects (meaning the drugs to treat such indications are predominantly of low commercial value), the European Commission places high importance on encouraging companies to invest in the research and development of orphan drugs.

However, timely and equitable access for patients to orphan medicines remains an issue. The review of the single medical research budget is a timely opportunity for the UK to take a lead within Europe on this issue, for the benefit of UK patients.

The BIA believes that there is an opportunity to build on the benefits already delivered by the National Specialist Commissioning Advisory Group (NSCAG), which was set up to advise Department of Health Ministers on which NHS services are best commissioned nationally, rather than locally, to ensure a high quality of clinical care and equity of access for patients as well as securing value for money. NSCAG’s remit covers ‘ultra-orphan’ diseases (less than 400 cases nationally).

The BIA recommends an increase in the NSCAG drugs budget to cover other disease areas and extending the NSCAG remit to cover orphan conditions in addition to ultra-orphan conditions.

- **Impact of VAT on the Use of Clinical Research Facilities for Collaborative Research Programmes with Industry Funding**

The BIA would like to draw attention to the VAT issues arising from the use of Clinical Research Facilities for industry-funded research in the UK. VAT imposes a block on collaboration either by restricting the level of collaboration activity or restricting the funds available for the university's contribution to the collaborative research.

We therefore support the following UK Clinical Research Collaboration's recommendations to:

- Introduce a refund scheme or a reduced rate of VAT specifically for research buildings operated by charities/universities/the NHS, and the research activities performed therein;
- Allow a fair and reasonable reclaim based on the actual activities, including the proportion originally ignored as being for non-business use; and
- Relax the 10 per cent business use restriction and ignore all educational functions provided to third parties.

- **Improving R&D tax credits**

The R&D Tax Credit scheme has been a valuable incentive to innovation that has been welcomed by the bioscience industry. The BIA would like to take this opportunity to make some proposals for further enhancements:

- extending the relief available for payments to clinical trials volunteers to cover to ensure the benefits are delivered directly by the company developing the medicine; most payments are made via an intermediary such as a CRO, so it is only those organisations that benefit from the relief; and
- extending the effect of the PAYE/NI cap where a company makes extensive use of subcontractors or externally provided workers, as this disadvantages companies who focus on making the best use of their skills and the skills of third parties in conducting their R&D activities.

The BIA would also like to recommend facilitating training on the bioscience sector for HM Revenue & Customs staff where appropriate, e.g. a seminar programme with accreditation. This could provide Inspectors with a greater understanding of how biomedical research companies operate at a technological level, to better inform their enquiries.

- **Research into analytical science and metrology for the UK biopharmaceutical sector**

In order to ensure its relevance to UK medical research and the improvement of national health and wealth, publicly funded research into analytical methods for biopharmaceuticals should:

- be integrated with other research such as bioprocessing; and
- include collaboration with industry and MHRA and NIBSC.

- **Skills and Training**

Developing, attracting and retaining a high quality scientific and managerial talent base are vital if we are to maintain sustainable competitive advantage in the UK bioscience sector.

The following issues are key:

- Supporting the building of the interdisciplinary talent pipeline; and
- Attracting and retaining the best current talent.

A recent survey of BIA members highlighted concerns including limited opportunities for post/graduates without industrial experience in bioscience SMEs; candidate quality and the need for greater appreciation of functional skills such as project management, regulatory affairs and quality.

The BIA is undertaking a significant amount of skills-related activity, including developing an in depth understanding of the bioscience and manufacturing interface through bioProcessUK; piloting a flexible Management Development Programme and running tailored careers roadshows.

The BIA is also involved in the SEMTA Bioscience Sector Strategy Group (SSG), although no significant progress has been made since the SSG's inception in 2003. One solution would be to take a new imaginative approach and move the funding for the SSG under the single medical research budget. This would need to be properly managed and taken forward in partnership with all stakeholders, including industry.