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5 August 2013

RE: Review of the balance of competences: research and development

Key points:

- **The UK is a European leader in many research and technology areas, notably the life sciences. To ensure the UK remains competitive on the global stage and is not held back by EU policy, it is important for the UK to engage effectively in the policy-making process.**
- **Given the UK philanthropic model of funding research is not replicated in all other EU nation states, the UK government may need to work harder to explain and champion the role of charities in medical research funding in the EU.**
- **The EU can play a powerful role in making the UK an attractive place for global investors in medical research; it does this through taking a leading role in international policy areas, harmonising research regulation and governance across the EU, promoting collaboration, providing funding and attracting a highly-skilled workforce.**
- **Medical research charities are keen to engage with the EU and have valuable insights into policy development. However there are challenges that both charities and the EU must overcome. The policy making process is often slow with multiple stakeholders and engagement is a challenge for organisations with limited resources. Unintended impacts of policies seem slow to be remedied.**
- **Increased transparency of EU processes and well-publicised consultations would facilitate participation, leading to more effective research and innovation proposals and earlier identification of unintended impacts.**

1. The Association of Medical Research Charities is a membership organisation of the leading medical and health charities funding research in the UK and overseas. We welcome the opportunity to respond to this consultation. Our vision is charities delivering high-quality research to improve health and wellbeing for all. Securing the best environment for medical research in the UK and EU is key to achieving this.

2. We will confine our remarks to those points most relevant to charity funders. We will begin by outlining how the EU influences the UK medical research environment. We will then outline future opportunities and challenges.

Impact on the national interest

3. **The UK must remain an attractive place to invest in medical research**
Our members invested over £1.2 billion into UK medical research in 2012, and approximately £137 million overseas.[1] The proportion of research investment going overseas has increased in recent years, from 6% in 2010 to 10% in 2012 , growing on average 15% per year. Research is international and medical research charities seek to fund the highest quality science wherever it is located. The UK science base must remain competitive on an international stage to attract public, private and charitable investment to maintain our uniquely-diverse medical research environment. Being an involved member of the EU is key to this.

4. The EU can have a positive impact on the UK medical research environment, including for example, by taking a lead in important international policy areas, harmonizing regulation, facilitating collaboration and providing funding and a skilled workforce. However, there are also examples where the EU has had a somewhat negative impact through the introduction of unnecessary bureaucracy and inflexible regulation. And where such impacts occur the process by which these can be addressed has been unclear and, even when clear, has been slow.

5. **Leading on policy issues**
The EU has a powerful leadership role and can catalyse action at both a national and international level. This is important where no one member state can lead, or where coordination across multiple countries or organisations is required.

6. **CASE STUDY:** Europe-wide policies and legislation have been implemented to support the development of "orphan drugs", which offer promising treatments for rare diseases but have a low commercial value.[2] Rare diseases are those that affect less than 1 in 2,000 people. Support for developers of orphan drugs includes market exclusivity, licensing fee reductions and R&D grants from the EU. The European Commission has also developed a Communication on Rare Diseases which sets out proposals for a comprehensive, EU wide strategy on issues including research, diagnosis, treatment and care for rare disease patients. This recommendation called on all EU member states to develop plans or strategies for rare diseases by 2013 to increase integration of strategies across Europe. These policies have build on the strength of the EU to bring large populations together and have galvanised activity across the EU to the benefit of people with rare diseases, researchers and drug developers in the UK.

7. **CASE STUDY:** Given the international nature of research, and in particular clinical trials, an internationally consistent approach to the registration, publication and sharing of clinical trial results and data is required if we are effectively to improve transparency of research

[1] 2012 AMRC research expenditure database - http://www.amrc.org.uk/our-members_charityfunded-research
[2] AMRC, *Opportunities for medical research charities to engage with Europe* (2011) http://www.amrc.org.uk/news-policy--debate_engaging-with-europe

outcomes. Without this, any action will have limited effect and serve to drive research activities elsewhere. The EU could take a valuable leadership position in this policy area on the global stage, and there are already signs of steps towards this[3]. This process led by the EU allows all member states to influence what global best practice looks like and ensure Europe is not disadvantaged. However to achieve this it must be conducted with effective engagement from all the stakeholders involved.

8. Harmonisation

Effective legislation can have a positive impact on UK medical research through the harmonisation of regulation and procedures across the EU. This promotes collaboration, the sharing of resources, increasing the efficiency of the research environment making the UK a more attractive place to invest. However, it is critical that all stakeholders are involved in the development of harmonisation initiatives to avoid any unintended negative impacts. As outlined below, unintended consequences can be barriers to research and the relatively slow process of EU legislation can result in long delays before these can be addressed.

9. Harmonisation can also be achieved outside of legislation, particularly through the collaboration of interested parties or competent bodies of member states.

10. **CASE STUDY:** Directive 2010/63/EU on the protection of animals used for scientific purposes[4] is expected to have a positive impact on UK research by harmonising regulation across Europe. This will bring welfare standards up to the levels in the Animals (Scientific Procedures) Act 1986 and ensure the UK is not at a competitive disadvantage compared to other member states. The UK is widely recognised as a world leader in the welfare of animals used in research and was instrumental in the preparation of the EU Directive.

11. **CASE STUDY:** The 2004 European Tissue and Cells Directive[1], as transposed into the Human Tissue Act 2004[2], takes a blanket approach to studies using tissue for human use and does not take in to account the considerable variability that comes with stem cell research studies, which has led to a negative impact on some UK research. A trial of cell transplantation for Parkinson's and Huntington's has experienced severe delays due to inflexible regulation under the Directive. The cell transplantation trial involves collecting foetal tissue from women undergoing abortions. Following informed consent they would undergo a blood test in advance of their procedure to identify any abnormalities. However, the Directive brought in the specification that blood tests had to be carried out on the day of the procedure. This would not allow sufficient time to conduct the appropriate tests and therefore has no safety benefits. Therefore, to comply with the regulation and ensure safety benefits are in place, blood tests would have to be taken in advance (for the purposes of the research) as well as on the day of the procedure (for the purposes of the regulation), causing extra unnecessary stress for the women involved. The Human Tissue Authority has no authority to overcome these issues. This has now caused the study to be halted even further whilst ethical approval is sought to carry out the additional blood test.

12. Promoting collaboration

[3] http://www.ema.europa.eu/docs/en_GB/document_library/Other/2013/06/WC500144730.pdf
[4] http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2010:276:0033:0079:EN:PDF

Collaboration at an international level benefits medical research by creating a critical mass and allowing the sharing of ideas, techniques and approaches. Many of our members value the role of the EU in facilitating collaboration, often through the provision of funding and also providing an impetus through policy leadership.

13. **CASE STUDY:** EFACTS (the European Friedreich's Ataxia Consortium for Translational Studies) promotes a collaborative pan-European translational research strategy for the rare autosomal recessive neurological disease, Friedreich's ataxia (FRDA). It is funded through a €6 million (£4.8 million) grant from FP7 (Health: Pre-clinical or Clinical research in rare diseases). The project has succeeded in recruiting the target of 600 patients with FRDA from across Europe, a great achievement for a rare disease. This project has involved a large natural history study and has allowed the testing of ataxia rating scales and the collection of samples to help in the search of biomarkers. The researchers have collaborated with the charity Ataxia UK, as well as other European ataxia charities, having a positive impact on FRDA research and patients with the condition in the UK.

14. EU funding streams

The continuation of the Horizon 2020 programme to continue support for collaborative research and innovation across Europe is very welcome. Larger medical research charities report that they have had positive experiences accessing EU funding, which is a valuable and complementary source of support for their researchers. However, European funding processes are complex and many small charities with valuable project proposals have little resource to invest in understanding the system and find it hard to participate. Charities tell us:

- The application process is protracted and overly complex. For example, an application for FP7 funding made by a consortium of Alkaptonuria (AKU) researchers took approximately 18 months and involved a 110-page application form[5]
- The European Commission is overly bureaucratic; decisions are slow to be communicated to partner organisations and reporting systems are burdensome.
- Funds are released periodically, meaning partners have to cover large up-front costs themselves while they wait up to a year for reimbursement. This creates unnecessary financial pressures especially for smaller research organisations.

15. Bodies established to help navigate the system – such as the National Contact Points – are welcome but are not very visible to those new to the system. It is notable that universities have become very successful at securing EU funds but only through employing staff specifically to support researchers through the process. There is a role for umbrella organisations such as AMRC in demystifying the system, and AMRC has ongoing work in this area. But it is important that accessibility of funding is considered as research and innovation proposals are developed in Europe, how this can be improved is discussed further below.

16. **CASE STUDY:** The AKU Society[6] works internationally to enable research into the rare disease Alkaptonuria (AKU). With 81 affected individuals identified in the UK and only a further 325 across the rest of Europe, an international collaborative approach is the only way to recruit enough participants for the study of the disease and to test potential treatments. The Society is

[5] Evidence provided by the AKU Society, which is a registered charity but not currently a member of AMRC.
[6] The AKU Society is a registered charity but not currently a member of AMRC.

leading a public-private consortium (including patient groups, hospitals, universities, industry and independent labs) drug trial of nitisinone in AKU patients, funded through a €6 million (£4.8 million) grant from FP7 (Health: Pre-clinical or Clinical research in rare diseases). This exemplifies the strength of charities in coordinating research for the benefit of patients, often in areas of high unmet need, but also demonstrates the positive impact the EU can have by providing financial support for projects that are prohibitively expensive for charities to undertake alone and too niche to attract national funders.

17. **Free movement of workers**
The ability of scientists and clinicians to move freely between member states is beneficial to medical research in the UK. As a scientific powerhouse within Europe our research institutions and businesses have considerable pull to attract the brightest minds from across Europe. Such movement also bring other benefits, promoting the sharing of ideas and new techniques and opening doors to further collaborations. The EU should continue to promote the free movement of such skilled workers.

Future opportunities and challenges

Working more effectively together

18. As described above, EU legislation, communications, initiatives and programmes can benefit the UK: its researchers, citizens and the economy. The EU can galvanise activity enabling greater investment and coordination which benefits UK research. Consistent and proportionate regulation is attractive to global investors.

19. However there are challenges. The often slow pace of EU policy-making involving multiple stakeholders can limit the ambition of resulting policy. The considerable resources required to engage with EU institutions can act as a barrier to some of those policy will impact on, limiting the EU's ability to identify adverse impacts before implementation. This may also prevent an organisation accessing initiatives that are intended to support them. The UK is recognized for its expertise in many policy areas and therefore has a deserved strong influence on EU policy. It is important that we use this strength, and where others are experts build on their best practice, to ensure that the EU does not prevent the UK from competing on a global stage but opens the doors to enable us to do so.

20. Medical research charities with their unique links to both patient groups and researchers, have valuable insights into unmet need and the actions needed to address health problems. They are keen to work with the UK government and the EU. Their engagement in policy making can avert unintended consequences and ensure policy delivers for the public, clinicians and researchers. However monitoring and inputting into EU policy development is a resource-intensive activity that many are unable to undertake individually. There is a role for umbrella groups such as AMRC and also for the government to facilitate this engagement but there are also a number of steps EU institutions, or national representatives in some cases, could take to make it easier for stakeholders to engage:

    - **Communication** of policy areas that the Commission intends to look at, and of the purpose of proposals at their outset, will allow charities to be better prepared to respond to consultation, development and implementation.

- **Increased transparency** to make it easier to follow policy-making processes and see where, when and with whom to engage (case study 3). This should include being open to engagement with national organisations.
- **Impact assessment** to identify where legislation in one field may impact on others and ensure that relevant stakeholders are consulted.
- **Realistic consultation timeframes and engagement** to ensure relevant stakeholders are aware and are able to respond.
- **Responsiveness** to address changes in the research environment.

21. Early engagement could prevent poorly considered legislation and speed the remedying of any unintended consequences which may hamper UK and EU competitiveness.

22. **CASE STUDY:** The Clinical Trials Directive 2001/20/EC[7] has created delays in trial setup due to inconsistent implementation of the Directive by member states, increased bureaucracy and inflexible regulation. Cancer Research UK coordinated a joint position across UK, pan-European and other European organisations to demonstrate a common position shared by the medical research community on proposals by the Commission for a new EU Clinical Trials Regulation.[8] The response to this has been positive and so far, helpful. The Commission ran several consultations on plans to revise the 2001 Directive and associated guidance and the draft legislation, published in July 2012, showed they listened to the concerns and viewpoints that were raised in the joint statement.[9] As the legislative process is ongoing, the UK medical research community is continuing to work with the UK government, MEPs, the Commission and counterparts elsewhere in Europe with the aim of ensuring that an effective and proportionate Regulation is agreed. This effective interaction is an example how national organisations can engage effectively with the EU.

Future challenges

23. The following areas have been identified by our members as of particular importance to medical research in the UK, this is not however exhaustive. The EU is a major influence in these areas and is in the position to not only lead Europe, but give EU member states a strong global voice.

24. **Shaping regulation**
Medical research is evolving rapidly. The processes of research regulation and licensing must adapt to enable us to trial new treatments on smaller populations and facilitate earlier access to life-saving treatments. The UK is already considering these issues at a national level, as are our international competitors in the US and Asia. The EU is currently taking a valuable lead through the European Medicines Agency (EMA), bringing together multiple stakeholders to shape EU policy in this area. If achieved successfully, the EU can become a fertile environment for developing the most innovative treatments, drawing in global investors. The UK can valuably engage with these initial steps to shape future policy and ensure we are well-placed to compete internationally.

25. **Research funding**

7 http://www.eortc.be/services/doc/clinical-eu-directive-04-april-01.pdf
8 http://www.cancerresearchuk.org/prod_consump/groups/cr_common/@nre/@pol/documents/generalcontent/cr_077460.pdf
9 http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=COM:2012:0369:FIN:EN:PDF

Our members have expressed concern that if European research priorities change or there is a severe reduction in funding available, valuable projects such as those described in our case studies will not be able to continue and progress will be lost. Our members are keen to engage with the EU to ensure that this does not happen, and AMRC will work with them to do this.

26. **Data protection**
Personal health records are a valuable resource for clinicians and researchers alike. The information contained within them can reveal the most effective ways to care for someone and allow us to better understand the causes and frequency of disease. The Data Protection Regulation currently under debate in Europe will impact on UK researchers who use personal data for research, including those that access NHS patient data. This may also impact on the government's own initiatives; the *Strategy for UK Life Sciences*[10] included a £60 million investment to establish a new secure data service called the Clinical Practice Research Datalink to service the needs of the research and life sciences community. Medical research organisations in the UK have raised concerns at a UK and EU level[11]. UK representation on the Council of Ministers scrutinising this Regulation is being led by the Ministry of Justice. It is important that the full impact on UK medical research and innovation are raised in negotiations in Europe.

27. The UK has a world-leading position in the life sciences. We have demonstrated how the UK benefits from membership of the EU and where there are further challenges. We have suggested some steps that could be taken to address these. It is important that the UK remains a strong voice in EU policy making to ensure our research is not hampered by EU activity but boosted by the EU's position on the global stage, enabling us to attract inward investment for medical research that will benefit UK health and wealth. We would be happy to expand on any of the points raised in this response.

Yours sincerely,

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[10] BIS OLS *Strategy for UK Life Sciences*, 2011
https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/32457/11-1429-strategy-for-uk-life-sciences.pdf

[11] Joint statement on the draft European Data Protection Regulation http://www.wellcome.ac.uk/About-us/Policy/Spotlight-issues/Personal-information/Data-protection-legislation/index.htm