Influencing and shaping our sector – BIA update January – April 2019

UK BioIndustry Association

Influence, connect, save

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Introduction

The BioIndustry Association (BIA)'s ongoing engagement enables our members' voices to be heard at the highest levels. This quarterly update gives an overview of key policy developments and the BIA's continued engagement with policymakers, regulatory authorities and wider stakeholders on behalf of the UK life sciences industry, from January to April 2019.

Brexit has moved at pace this quarter. While the withdrawal date has been extended until October this year, a no-deal scenario remains a possibility. We are continuing to work with government, industry, and stakeholders on a range of issues from no-deal planning and medicine regulation, to intellectual property and future trade.

However, despite the uncertainty of Brexit, our finance data for the first quarter showed that biotech companies got off to a strong start in 2019 and raised £182m in venture capital. We have already started to highlight this positive news to the Government and UK Research & Innovation as part of our campaign for the forthcoming Spending Review. The BIA is also continuing to engage with the Government at the highest levels through the Life Sciences Industrial Strategy Implementation Board and we organised a member workshop on rare diseases. Read about this and much more below.

This quarter in numbers:

15+ influence meetings with 12+ MPs and Peers, including 5 Ministers

8 consultation responses submitted

8 letters to Ministers

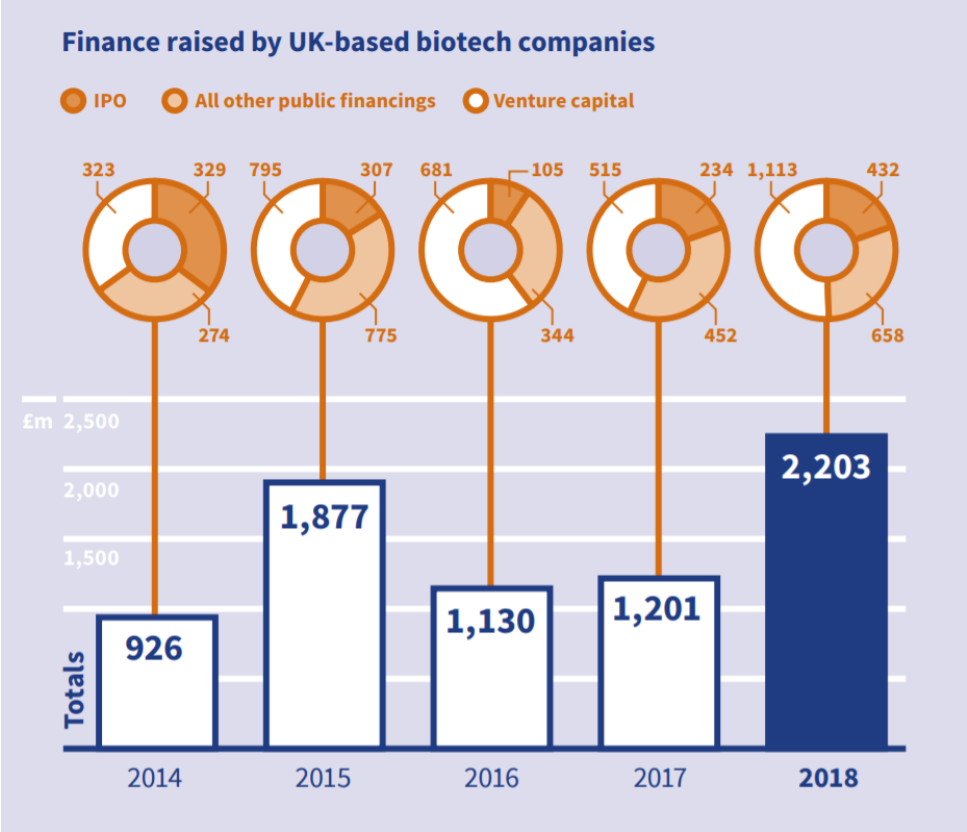
Contents

Engagement with the Government on life sciences policy

The BIA continues to engage with the Government on life sciences policy through the Life Sciences Council and its working subgroups. One of these subgroups is the Life Sciences Industrial Strategy Implementation Board, which met in March. The meeting provided an opportunity to discuss the progress made on the implementation of the two Sector Deals with Life Sciences Minister Lord Henley, Innovation Minister Baroness Blackwood, industry stakeholders and relevant government departments.

At the meeting, the Office for Life Sciences (OLS) and the Department for International Trade (DIT) explained that they are developing a 'Life Sciences Global Sales Pitch', a project aimed at promoting the UK life sciences offer globally. As well as setting out the overarching UK offer, the project will also develop individual pitches on key areas where the UK can differentiate itself from competitors. During the discussion, the BIA's report *Confident capital: backing UK biotech*, which revealed that the UK biotech sector raised a record £2.2bn from investors in 2018 – was held up as an illustration of our sector's success and competitiveness.

Increasing SMEs' access to finance is a key part of building on the sector's competitiveness and a core focus of the Life Sciences Industrial Strategy. As the Patient Capital Review is nearing its conclusion, and with the British Business Bank keen to work with the sector, the Board heard that the Government will develop this area of work further this year. The Board discussed the importance of enabling pension funds to invest in early-stage companies, such as biotech SMEs. The BIA has been engaged with this agenda for many years and we are pleased that the Government and regulators are now taking steps to unlock pension fund investment.



The biotech sector's strong finance performance in 2018 was held up as an example of the sector's competitiveness at a recent Life Sciences Industrial Strategy Implementation Board meeting.

Leaving the EU

General Brexit update

At time of publication, the EU and UK have agreed an extension to Article 50 that may last until 31 October. However, if the Withdrawal Agreement is ratified by both parties before this date, the withdrawal will take place on the first day of the following month. If the UK is still a Member of the EU at 23-26 May 2019 and if it has not ratified the Withdrawal Agreement by 22 May 2019, it must hold EU elections (and if it does not hold elections, the withdrawal date will be 1 June). The Withdrawal Agreement cannot be reopened for further negotiations, but the EU will reconsider the Political Declaration on the future relationship.

The BIA continues our government and ministerial engagement via the EU Relationship Group, which has met several times this year. We also continue to brief parliamentarians about the need to avoid a no-deal Brexit and advocate industry policy priorities – regulatory cooperation, frictionless trade, movement of talent, R&D cooperation and funding. We are also ensuring that we are ready if the Withdrawal Agreement is passed to seek an early deal in the future relationship for medicines and patients.

BIA launches new Brexit microsite

In February, the BIA launched a new Brexit microsite to provide a single location for our members with information from the BIA as well as updates from the Government and the EU. The BIA regularly receives government updates and adds them to the microsite. We aim to point our members in the right direction for the right information – whether it is for no-deal planning, the extension, the Withdrawal Agreement or the future UK-EU relationship.

BIA continues to work with government on medicines supply contingency planning

The BIA has continued its work with the Government on the Medicines Supply Contingency Planning Programme, which provides guidance for marketing authorisation holders in the event of a no-deal Brexit. Despite the Brexit extension to 31 October, a no-deal Brexit is still a possibility and therefore the programme continues. As the Government considers its requirements of industry with new possible dates for a no-deal Brexit, the BIA continues to feed into discussions that a heightened state of readiness is not sustainable for companies. We have informed them that it is an impossible task for industry to constantly be ready for a no-deal Brexit and that industry needs to know what the Government requires of companies.

BIA briefs parliamentarians on no-deal medicines and clinical trial statutory instrument

The BIA briefed MPs and Peers as the medicines regulation and clinical trial statutory instruments undertook their passage through Parliament this quarter. The statutory instruments will come into force if the UK leaves the EU without a deal to allow for the continued sale of, and access to, medicines, medical devices and clinical trials for the benefits of UK patients. While the BIA highlighted several concerns in our briefings to parliamentarians with the regulations, and called for immediate review of the statutory instruments if they should come into force, we advocated for the approval of the statutory instruments.

The statutory instruments the Human Medicines (Amendment etc.) (EU Exit) Regulations 2019, the Medical Devices (Amendment etc.) (EU Exit) Regulations 2019 and the Medicines for Human Use (Clinical Trials) (Amendment) (EU exit) Regulations 2019) have now all been approved by Parliament and will come into force on the day of EU withdrawal in the event of a no-deal.

BIA welcomes Minister's reassurance on patents statutory instrument

The BIA provided briefings to Peers as the no-deal Brexit statutory instrument Patents (Amendment) (EU Exit) Regulations 2018 was tabled in the House of Lords. We raised concerns in our briefings over the statutory instrument, which would in effect mean that, in the event of a no-deal Brexit, supplementary protection certificate (SPC) holders would enjoy a shorter period of protection in the UK than in the EU unless their UK Market Authorisation was awarded before or at the same time as the EU-wide Market Authorisation, which would be unlikely in such a scenario. This would only impact SPCs awarded after the UK left the EU but signifies a worrying erosion of IP protection by the Government, which would be of concern to industry and global investors.

The BIA raised concerns that the statutory instrument would send a signal globally that the UK Government may not be fully committed to a gold standard intellectual property regime, and that it will in turn impact the attractiveness of the UK as a location for international investment. We are also concerned about the lack of formal stakeholder consultation on the statutory instrument. Our concerns were highlighted by Lord Warner, a former health minister, during the debates in the Lords. The statutory instrument was initially 'negatived' (rejected) but returned to the Lords where Life Sciences Minister, Lord Henley made several reassurances to the sector, including committing to a review of the regulations introduced by the statutory instrument if there was a no-deal Brexit and the statutory instrument entered into force. Furthermore, Lord Henley stated that if there is a no-deal Brexit, the Government will immediately start to explore the future landscape with our sector.

Ongoing BIA Brexit activity – Brexit lead network events and webinars

In February and April, we held Brexit Lead Network events together with the ABPI. The events have a member only session to discuss industry views, as well as a government update from senior civil servants. In February, Patrick Carey, Deputy Director, EU and International at the MHRA spoke about their no-deal planning and Steve Oldfield, Chief Commercial Officer at the Department of Health and Social Care gave an update on Brexit Medicine Supply Contingency Planning Programme.

In April, we heard from Syma Cullasy-Aldridge, Head of Business and International Engagement at Department for Exiting the EU; Matt Porter, Engagement Adviser, EU Exit Immigration Strategy at the Home Office; and Steve Oldfield returned to give another Brexit Medicine Supply Contingency Planning Programme update. There will be three further events this year and BIA members can register for free on our website.

We also continue to hold our free monthly webinars where our CEO Steve Bates and Brexit lead Laura Collister explain the latest Brexit updates and what they mean for the sector. Register for the next webinar on our website or tune into past webinars on our YouTube page.

Medicines Regulation

UK contingency legislation and MHRA guidance for the regulation of medicines, clinical trials and medical devices

To support industry preparations for a no-deal EU exit, the MHRA is publishing a series of guidance documents on operational changes, covering their proposed arrangements for the regulation of medicines, clinical trials and medical devices if the UK leaves the EU without a withdrawal agreement. These can be found here.

Contingency legislation is also needed to enable the MHRA to take on regulatory processes that are currently undertaken by the EMA and other bodies in the event of a no-deal Brexit. Following a public consultation by the MHRA last October, to which the BIA provided input through a joint submission developed in collaboration with the ABPI, the medicines, medical devices and clinical trials no-deal statutory instrument were laid in Parliament in late January 2019.

The BIA advocated for the approval of these statutory instruments which will allow for the continued sale of, and access to, medicines, medical devices and clinical trials for the benefits of UK patients. They have now all been approved by Parliament and will come into force on the day of EU withdrawal in the event of a no-deal (see page 6 for more).

DHSC clinical trials contingency planning

In February, the BIA held a meeting with officials from the DHSC to discuss clinical trial supply in a no-deal Brexit scenario. The meeting provided the opportunity for the BIA to get an update on the outcome of the DHSC 'deep dive' survey on clinical trials conducted earlier this year. It was also an opportunity for DHSC to hear from BIA members how clinical trial supply chains operate currently as part of their 'business as usual' activities and to better understand the contingencies members have put in place, both as part of 'business as usual' and as new contingencies being put in place ahead of EU exit to ensure continuity of supply.

In March, the DHSC issued a communication regarding the National Supply Disruption Response (NSDR) processes established by the DHSC, together with some additional information from DHSC and other government departments relevant to clinical trials and clinical investigations in the event of the UK leaving the EU without a deal. These documents can be found on the BIA Brexit website. For queries to the DHSC Clinical Trials Contingency Planning Team, please contact ctcontingencyplanning@dhsc.gov.uk.

EU actions to prevent medicine shortages

In March, the EMA Management Board noted that the number of EU centrally authorised medicines at risk of shortages continues to decrease as more companies are taking the necessary steps to ensure that their medicines can remain on the market.

On 26 March, the EMA released a Q&A document for patients, healthcare professionals and the general public on the preparatory work that EU authorities are doing to prevent medicines shortages. Safety and evaluation of medicines, clinical trials and supplies are topics addressed in the Q&A.

EMA and European Commission update Q&As and guidance to help companies prepare for Brexit

The BIA has continued its engagement with EU regulators and participated in the last EMA Industry Stakeholder meeting on Brexit and operation of the centralised procedure before the EMA left the UK on 1 March 2019. The presentations from the meeting are available on the EMA website.

In February, the EMA and the European Commission published updated Q&As related to centrally authorised medicines in the event that the UK leaves the EU without a withdrawal agreement. The added Q&As address supervisory authorities for manufacturing sites; GMP certificates issued by UK authorities; Falsified Medicines Directive safety features; parallel distribution notices; reporting requirements into EudraVigilance concerning data from UK; and the impact on on-going referral procedures involving the UK. The Q&As and Brexit-related guidance can be found on the EMA website.

Subsequently, the European Commission published a communication on possible exemption to the rules for batch testing of medicinal products. On 21 February, the Commission's Directorate General for Health and Food Safety (DG SANTE) sent a letter to the EU27 Heads of Medicines Agencies and to the EMA's Executive Director which specifies that marketing authorisation holders who are unable to transfer their batch testing site from the UK to the EU27 by the Brexit date may be permitted, for a limited period of time, to rely on quality control testing performed in the UK under certain conditions. This will help to lower the number of products currently considered at risk of supply shortages.

The EMA released this video looking back on their time in London from 1995 to 2019 and thanked the UK and London community for their gracious hosting. The BIA wishes the Agency well as they settle in their new space in Amsterdam, the Netherlands, and become fully operational.

After 24 years, the EMA closed the doors of its London offices on 1 March 2019.

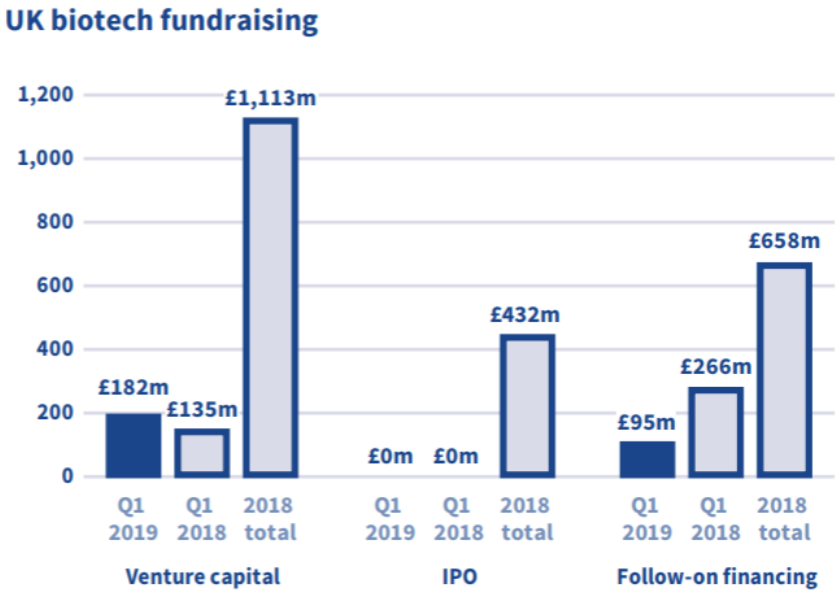
BIA Brexit website – Regulation & IP

The UK Government and its agencies, including the MHRA, have published and continue to publish information on contingency planning for the regulation of medicines, clinical trials and medical devices if the UK leaves the EU with no-deal. Guidance documents and information from the MHRA and DHSC, the EMA and European Commission on Brexit preparations and how they will operate in a no-deal scenario are being added regularly to our Regulation and IP page on the BIA Brexit website to keep members informed with the latest developments. For more information please contact Dr Christiane Abouzeid at CAbouzeid@bioindustry.org.

Finance, tax and investment

Investors maintain confidence in UK biotech at start of 2019

The BIA and Informa Pharma Intelligence released figures in March that showed a strong start to 2019 for venture capital investment in UK biotech. £182m was raised by private biotech companies between December 2018 and the end of February 2019, almost £50m more than during the same period a year earlier. The figures suggest that the sector could break the fundraising record set in 2018, when over £1bn was raised in venture capital.



Data from the BIA shows that UK biotech companies got off to a strong start in 2019.

BIA calls for rule changes to promote pensions investment in biotech

The BIA has responded to four consultations urging regulators and the Government to promote greater investment by pension schemes into venture capital, including biotech. The BIA submitted views to three Financial Conduct Authority (FCA) consultations on rules related to the operation of investment funds and restrictions on what types of illiquid assets pension funds can invest in. We also submitted a response to a consultation run by the Department for Work and Pensions on incentives that would encourage pension trustees to invest in venture capital. The submissions are part of a wider body of work the BIA is conducting to unlock pension fund capital for the sector.

Proposals to cap SME R&D tax credits published

The Treasury and HMRC have published a consultation on the proposed cap on cash payments made to SMEs through the R&D tax credits scheme. The cap would limit cash payments to three-times the company's PAYE and social security liabilities; it is intended to reduce fraudulent claims. The BIA met with government officials in January to provide evidence that this will inadvertently impact genuine biotech companies. Following that representation, the consultation has been published with further measures intended to reduce unintended consequences for genuine companies, but they do not go far enough. The BIA is currently collecting data to inform our policy response and contacting MPs to raise our concerns. For more information please contact Dr Martin Turner at mturner@bioindustry.org.

Engaging with China

Building a strong bridge between the UK and Chinese life sciences ecosystems remains a core priority for the BIA throughout 2019. In March, AstraZeneca announced that it is spearheading the creation of the new International Life Science Innovation Park in China, which will be built by the Wuxi municipal government and Wuxi High-tech District. BIA CEO Steve Bates was present at the announcement and the BIA co-signed a Memorandum of Understanding, where we pledged to help establish UK life science companies in Wuxi. Steve commented:

> *"China is an important and exciting opportunity for UK life science companies. The chance to participate in the creation of the new Wuxi cluster is one not to be missed. This new opportunity will enable UK companies to co-locate with AstraZeneca in China, within a truly supportive local ecosystem, making it easier for UK life sciences companies to partner, grow and innovate with like-minded dynamic companies and investors in China."*

Steve also attended the inaugural Wuxi Healthcare Forum in Shanghai where he joined a panel of experts to discuss the opportunities for life science companies in both the UK and China. See our blog for Steve's analysis on the changes China has made to improve healthcare in the nation and attempt to become a leader in biotech.

BIA CEO Steve Bates speaking on a panel at WuXi Healthcare Forum in Shanghai.

Chancellor delivers Spring Statement with little fanfare

In his Spring Statement in March, Chancellor Philip Hammond committed £45m to the European Bioinformatics Institute near Cambridge to support genomics research, £79m for a super computer in Edinburgh, and £81m for a laser facility in Oxford, which will, among other uses, be used for molecular imaging of drug targets.

The statement was largely overshadowed by a rapidly-approaching (but since extended) deadline for the UK to leave the EU. However, it did confirm there would be a Spending Review this year (see page 12) and changes to immigration rules for researchers (see page 15). The BIA blog provides a full report on the Spring Statement.

Ensuring the voice of biotech SMEs is heard in the 2019 Spending review

This year, the Government will (subject to Brexit) conduct a Spending Review (SR), which is the process by which it is decided how much each government department will spend in the next three years. This has a major impact on our sector as public funding bodies, such as UK Research and Innovation (UKRI), its Councils, Innovate UK and the National Institute for Health Research (NIHR), are funded through government departments. The SR is therefore an important opportunity to demonstrate to the Government why more public investment into our sector is good for the health and wealth of the country, which is why the BIA's SR campaign is already in full swing.

Bringing the key messages to Parliament

In February, the BIA jointly organised an event in Parliament together with partner organisations in the sector through the All-Party Parliamentary Group (APPG) for Life Sciences and the APPG on Medical Research. The event brought together MPs and Peers, key civil servants, stakeholders from both industry and charities, and representatives from funders, such as UKRI and Innovate UK to discuss how cross-government coordination in life sciences is needed to deliver on the Government's commitment to reach 2.4% of GDP investment in R&D by 2027. We heard from Anneliese Dodds MP, Labour Shadow Treasury Minister, Chris Green MP, Chair of APPG on Medical Research, Barbara Domayne-Hayman, Entrepreneur in Residence at the Francis Crick Institute, and Jane Taylor, Chair of the Patient Insight Partner Group at Versus Arthritis. More information about the event is available on our blog.

Chris Green MP and Anneliese Dodds MP at the event in Parliament.

Discussing Spending Review priorities with funders and members

At our annual Committee Summit in February, we brought together a stellar panel in front of our wider membership to discuss what the life sciences sector can expect from the SR. The panel was chaired by Lord (David) Willetts, UKRI Board member and former Science Minister (2010-2014), and we heard from Alex Marsh, Deputy Director of Strategy at UKRI, Melanie Welham, Executive Chair at BBSRC, Emily Hirsz, Senior Policy Adviser at the Treasury, Mike Batley, Deputy Director of Research Programmes at NIHR, and Richard Hebdon, Head of Health & Medicine at Innovate UK. BIA CEO Steve Bates also presented the BIA's emerging key messages, which can be found on our blog here.

BIA-Government roundtable on R&D environment for SMEs

In February, the BIA also organised a roundtable with our SME members and the Office for Life Sciences (OLS) to discuss factors that affect SMEs' R&D investment. The meeting focused on challenges for life sciences R&D investment, supportive tax policies and suggested enhancements, and supportive R&D environment policies and proposed improvements.

The attendees were clear that the R&D tax credits system is the most efficient for delivering innovation support but highlighted that the proposals to introduce a 3X PAYE/NI cap on the SME R&D tax credits scheme will significantly harm R&D investment if enacted (see page 10). The attendees were also clear that sector-specific Innovate UK grant funding, such as the Biomedical Catalyst, must be maintained and expanded to enable early-stage companies to grow and leverage private investment. The roundtable will feed into the Government's thinking on how the UK will reach 2.4% of GDP investment in R&D by 2027.

BIA responds to Innovate UK proposal on repayable grants

In March, Innovate UK confirmed that it is considering repayable grants as a mechanism to anchor activity, investment and return in the UK. Under the proposal, grants would become repayable if companies go on to list on an overseas stock exchange or sell to foreign companies.

The BIA is strongly opposed to the proposal as it would send negative signals to foreign companies that their investment is not welcome in the UK, which is at odds with the Government's 2.4% target. In addition, a repayable grant would be treated as debt, which would make it more difficult to raise further equity. The proposal does also not reflect the fact that an IPO is not a final step and sign of success in biotech, nor is an M&A. The BIA is continuing to make these points in our ongoing discussions with Innovate UK and UKRI.

We conveyed our concerns about the proposal in a meeting with senior civil servants from the Treasury. In the meeting, we also discussed the Government's SR priorities, our key messages, the 3X PAYE/NI cap on SME R&D tax credits and the State Aid requirements for companies that are awarded grant funding.

Strategic technologies and areas of scientific focus

BIA continues to engage with Genomics England

Following the success of the 100,000 Genomes Project, Genomics England announced in January that Sir John Chisholm would step down as Chair. Sir John has been replaced by Jonathan Symonds. Genomics England also announced that its Chief Executive, Professor John Mattick, would leave the company. Its Chief Scientist, Professor Mark Caulfield is acting an interim Chief Executive. The BIA's Genomics Advisory Committee had a positive meeting with Professor Caulfield in February to discuss Genomics England's priorities, its recent work and how the company interacts with SMEs.

In March, the BIA attended the *Celebration of the 100,000 Genomes Project* at the Royal Society. The event celebrated Genomics England's remarkable achievement of sequencing 100,000 whole genomes, reviewed lessons learned and considered what the future holds for genomics.

Health Secretary Matt Hancock spoke at the event at the Royal Society and emphasised that genomics holds the key to new cures and treatments for patients in the NHS.

The BIA and several members also attended Genomics England's *Discovery Forum* at IQVIA. The Discovery Forum provides a platform for collaboration between Genomics England, industry partners, academia, the NHS and the wider UK genomics landscape, and it was particularly interesting to hear more about Genomics England's priorities in view of the recently announced National Genomic Healthcare Strategy. The BIA is working with the Government to ensure that the voice of genomics SMEs is heard as the Strategy is developed.

BIA members inform the formation of new Digital Innovation Hubs

The BIA is continuing to support Health Data Research UK (HDR UK) in the work to build new Digital Innovation Hubs for health research. This quarter, we have enabled our SME members to provide their expertise to inform the formation of the Hubs via regional workshops and one-to-one in-depth interviews.

The purpose of the Hubs is to connect health-related data of populations of around 3-5 million people within a single framework, allowing researchers and scientists to safely and securely use the data to develop new scientific knowledge and emerging technologies. If you would like to get involved in future engagement opportunities on the Hubs, please contact Eric Johnsson at ejohnsson@bioindustry.org.

Skills, people and talent

BIA runs programme for young entrepreneurs for the second year in a row

For the second year running, the BIA and Francis Crick Institute held our Programme for Up and coming Life Sciences Entrepreneurs (PULSE), a two-day workshop for aspiring entrepreneurs. We had eleven individual entrepreneurs from universities across the country and four researchers from the Crick. The workshop was held at the iconic Francis Crick Institute with the sessions run by experienced life sciences professionals and seasoned entrepreneurs, largely from BIA membership. The agenda covered the skills and knowledge needed to start and develop a successful biotech company, information sessions on topics ranging from the idea generation, business model planning and IP considerations through to interactive workshops on different equity funding models and dos and don'ts of business plan pitching.

This year's PULSE cohort at the Francis Crick Institute.

Apprenticeship programmes on advanced therapies continues to push ahead

During the National Apprenticeship Week in March, the Advanced Therapies Apprenticeship Community (ATAC) held its second annual meeting. Apprentices and participating employers gathered at Oxford BioMedica to discuss how the apprenticeship programme is key to meeting the industry demands for advanced therapies. Dr Ian Campbell, interim Executive Chair, Innovate UK, delivered the keynote speech and highlighted the Government's commitment to the programme. Three new programmes were also launched: regulatory affairs, senior management (MBA) and a Scottish Modern Apprenticeship in Life Sciences.

In addition, the first ATAC programme – a Level 5 Advanced Therapies Scientist standard that started in September 2018 - is running again this year and now recruiting. If you would like to know more, see the ATAC website or contact Netty England at aengland@bioindustry.org.

Immigration restrictions relaxed for researchers

From Autumn 2019, PhD-level occupations will be exempt from the Tier 2 (General) visa cap, and the immigration rules on 180-day absences will be updated so that researchers conducting fieldwork overseas are not penalised if they apply to settle in the UK. The announcement follows the BIA's joint lobbying on the issue, led by the Campaign for Science and Engineering.

Intellectual property and technology transfer

Supreme Court provides welcome clarity following BIA intervention

In a ruling handed down on 27 March, the UK Supreme Court confirmed that discoveries made through well-established or routine research protocols can be inventive and rewarded with a patent.

The BIA welcomed the clarity provided by the decision, having intervened in the case to ask that the Supreme Court not give a decision that could have unintended adverse consequences for patents for inventions made during the pre-clinical or clinical trial process. We argued this would significantly raise the hurdle for companies to attract the investment needed to identify and develop new medical innovations.

IPAC discusses key sector issues with government

In the last quarter, the BIA's Intellectual Property Advisory Committee (IPAC) has been busy meeting with government officials to discuss a range of issues on behalf of the sector.

A bilateral meeting with the Intellectual Property Office (IPO) took place on 25 February, where IPAC members shared views on the no-deal planning legislation passing through Parliament (see page 7), along with other Brexit matters, and a range of court cases currently being considered by the Court of Justice of the European Union.

IPAC also represented the sector on the Department for International Trade's IP Trade Policy Advisory Group, which is informing the Government's approach to trade negotiations with the US and other countries. In addition, representatives of IPAC attended an IPO stakeholder meeting on international patent law harmonisation.

Pre-clinical and clinical research

CTFG issues recommendation paper on the initiation and conduct of complex clinical trials

The Clinical Trials Facilitation and Coordination Group (CTFG), a working group of the Heads of Medicines Agencies, issued its Recommendation Paper on the Initiation and Conduct of Complex Clinical Trials in February, paving the way for a harmonised approach to the development of personalised medicines.

CTFG would welcome feedback on these recommendations. The guidance document outlines the key issues that sponsors should address when initiating and conducting complex clinical trials in the EU/EEA, and highlights differences between these trials and conventional clinical trials with regard to submitting clinical trial applications or requesting substantial amendments.

A clinical trial is considered to have a complex clinical trial design if it has separate parts that could constitute individual clinical trials and/or is characterised by extensive prospective adaptations such as planned additions of new Investigational Medicinal Products (IMPs) or new target populations. Examples of complex clinical trial designs are basket, umbrella and platform trials. These designs are commonly used in oncology but may be applied in other therapeutic areas if appropriately justified.

Development of the Clinical Trials Information System for the EU Clinical Trial Regulation

On 20 February, the EMA updated stakeholders at a meeting attended by the BIA on the status of development of the Clinical Trials Information System (formerly the EU clinical trial portal and database), which will be the single-entry point for clinical trial applications and supporting dossiers in the EU. The project plan was revised to improve delivery and to ensure that stakeholders can give feedback more regularly so that their expectations can be considered, whilst enabling the EU Clinical Trials Regulation to come into application as early as possible but retaining the possibility to extend functionalities in the future. Further announcements will be made before user acceptance testing commences on the release that will be subject to audit. The clinical trial system continues to be a priority in the EMA's Brexit preparedness business continuity plan.

Clinical trials and travel insurance

The BIA was asked by the Faculty of Pharmaceutical Medicine to raise awareness among our membership of a statement on clinical trials and travel insurance issued by the Academy of Medical Royal Colleges in agreement with the Association of British Insurers. The statement aims to reassure clinical trial participants that their participation in a trial would not affect their ability to get insurance beyond any medical condition they may have.

Manufacturing

Medicines manufacturing innovation in focus at Innovate UK and KTN event

In March, the BIA and many of our members attended an event organised by the KTN and Innovate UK, *Innovation in Medicines Manufacturing through Collaboration*, at the Centre for Process Innovation (CPI) in Darlington.

The event brought together attendees from industry, academia and funders to disseminate projects funded through the medicines manufacturing challenge in the Industrial Strategy Challenge Fund (ISCF). Since the ISCF was launched in 2017, the Government has been investing in a range of infrastructure and innovation projects in support of medicines manufacturing.

The event provided a great opportunity to learn more about a selection of projects that have been funded to accelerate the development and manufacture of vital advanced therapies, medicines and vaccines. We also heard the BIA's Manufacturing Advisory Committee and Science and Innovation Advisory Committee on how the UK's current capabilities in medicines manufacturing innovation can be enhanced.

The event on innovation in medicines manufacturing was held at the CPI's National Biologics Manufacturing Centre in Darlington.

Access to medicines

BIA responds to Parliamentary inquiry into the availability of Orkambi

In November 2018, the Health and Social Care Committee launched an inquiry in the availability of Orkambi on the NHS. The first hearing took place in March 2019, with the committee taking evidence from patient groups, representatives from NICE and NHS England, and finally Dr Jeff Leiden, Chair, President and Chief Executive of Vertex.

Ahead of the hearing, the BIA's Rare Disease Industry Group produced a submission to the committee, detailing industry's concerns around the appraisal of medicines for rare diseases.

During the hearing, it became clear that both committee members and NHS England had read the BIA's submission, with John Stewart, Acting Director of Specialised Commissioning, directly quoting from the document.

Following the session, Jeff Leiden met with the Secretary of State for Health, Matt Hancock MP, to discuss the impact. We understand that the meeting was productive and that discussions are continuing at official level.

BIA briefs MPs ahead of debate on NICE appraisals of rare disease medicines

In March, the House of Commons debated NICE appraisals of rare disease medicines, led by Liz Twist MP. The BIA produced a briefing for Ms Twist and other relevant MPs expected to speak in the debate. The briefing set out key concerns around the appropriateness of NICE's single technology appraisal (STA) and highly specialised technology (HST) processes to assess rare disease medicines and the need for flexibility to accommodate orphan and ultra-orphan therapies.

The debate focused on the impact on patients of being unable to access medicines, with many MPs citing anecdotal accounts from their own constituencies. Where there was detailed discussion of NICE appraisal processes, MPs highlighted the inflexibility and inappropriateness of the current appraisal process, in line with BIA's briefing to MPs.

The then Minister, Steve Brine MP, indicated the issues raised regarding the specific challenges faced by orphan and ultra-orphan medicines will be considered during NICE's upcoming methods review.

BIA hosts member workshop on rare diseases

In April, The General Managers of the members of the BIA's Rare Disease Industry Group (RDIG) met to discuss an additional workplan for 2019. This drew on discussions at the GM dinner in November 2018, where it was decided that RDIG should be more active in its engagement.

In particular, the GMs discussed how to engage with NICE's methods review later in the year, as well as wider engagement with relevant stakeholders, such Blake Dark, the recently appointed NHS Commercial Medicines Director at NHS England.

Building on the discussions at the workshop, the BIA is developing an action plan for RDIG for the year ahead and considering how best to implement new activities and stakeholder outreach initiatives.

For more information on the BIA's activities in policy and regulatory affairs, or to share feedback on this report, please contact Eric Johnsson, Senior Policy and Public Affairs Executive, on 0207 630 2197 or ejohnsson@bioindustry.org.

Not a BIA member? If you want to have a say on policy areas key to the life science sector, contact Michael McGivern, Membership and Business Development Manager, on 0207 630 2194 or mmcgivern@bioindustry.org

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BIA SUPPORTERS

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