Influencing and shaping our sector – BIA update October 2020 – January 2021

UK BioIndustry Association

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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 sector, from October 2020 to January 2021.

Towards the end 2020, the BIA celebrated the news that the Pfizer/BioNTech vaccine had received approval for emergency use in the UK by the MHRA. On the same day as vaccinations were rolled out across the UK, we held our annual MHRA-BIA bilateral meeting and hosted an All-Party Parliamentary Group (APPG) for Life Sciences briefing for MPs together with the ABPI and BIVDA on the life sciences industry's response to COVID-19. The last-minute Brexit deal, agreed on Christmas Eve, represented a culmination of many years of influencing work by the BIA and our industry partners. We promptly published an analysis of the deal's implication for life sciences and we are continuing to work with the Government to foster an environment that supports our sector.

We have also worked to influence the Government's priorities for the Spending Review by highlighting the importance of the Biomedical Catalyst and the availability of scale-up capital. As the Government seeks to increase its powers to scrutinise and intervene in investment deals through the National Security and Investment Bill, we are engaging with officials to minimise the negative impact of the Bill on our sector. We also responded to the long-awaited NICE Methods Review consultation, which will frame the medicines access landscape for many years in the future. Read about this and much more below.

This quarter in numbers:

16+ influence meetings with 18+ different MPs, Peers and MEPs, including 7 Ministers

11 consultation responses and briefings submitted

6 letters to Ministers

Contents

BIA engagement with the Government and Parliament on life sciences policy

The final quarter of 2020 saw the end of the transition period for the UK's exit from the EU, with a Trade and Cooperation Agreement (TCA) being secured at the last minute. As well as working with the Government on the impact on life sciences of this new relationship, the BIA maintained its focus on COVID-19 in the second wave of the pandemic and made representations to the Government's Spending Review about measures needed to support the UK's life sciences industry.

The **Life Sciences COVID-19 Response Group** continued its collaborative work as a ministerial meeting with industry, led jointly by Department of Health and Social Care (DHSC) Life Sciences Minister, Lord Bethell and Department for Business, Energy and Industrial Strategy (BEIS) Life Sciences Minister, Nadhim Zahawi.

This group is supported by officials from the Office for Life Sciences (OLS) in a COVID-19 Industry Group which convenes between the ministerial meetings. The joint government/industry testing webinar series has been replaced by a fortnightly Testing Taskforce meeting, convened by the DHSC's mass testing secretariat.

At the end of November, Minister Zahawi was appointed Vaccines Minister jointly in DHSC and BEIS, giving up most of his other responsibilities in BEIS but retaining his life sciences brief. In January, Alok Sharma took charge of preparations for the COP26 UN Climate Change Conference and was replaced as Secretary of State for Business by Kwasi Kwarteng. The BIA has written to the new Business Secretary setting out our industry's priorities for his department.

The BIA continues to support government-industry engagement through its membership of the **Life Sciences Council** (LSC) and the joint government-industry secretariat that coordinates the work of the Council and its sub-Councils and expert groups.

The Autumn meeting of the LSC was held virtually on 23 November, attended by Health Secretary Matt Hancock and Health Minister Lord Bethell with the then Business Secretary Alok Sharma, and Minister Zahawi. The meeting heard from Kate Bingham, Chair of the UK Vaccines Taskforce, about the impacts of the pandemic. Steve Bates responded for the BIA by welcoming the partnership between government, NHS, industry, academia and regulators in response to the pandemic and considering how these new ways of working could be embedded to build a thriving life sciences sector to aid the UK's economic recovery.

During the discussion on Brexit, Steve Bates raised concerns about the Northern Ireland Protocol and supply issues as well as re-stating the importance of a Mutual Recognition Agreement (MRA) for medicines regulation. BIA Board member, Dan Mahony contributed to the discussion of phase three of the implementation of the Life Sciences Strategy, focussing on the investment environment and potential for growth of innovative life sciences businesses of the future, stressing the importance of government activity on scale-up and explaining the continuum of investors involved in a growing company.

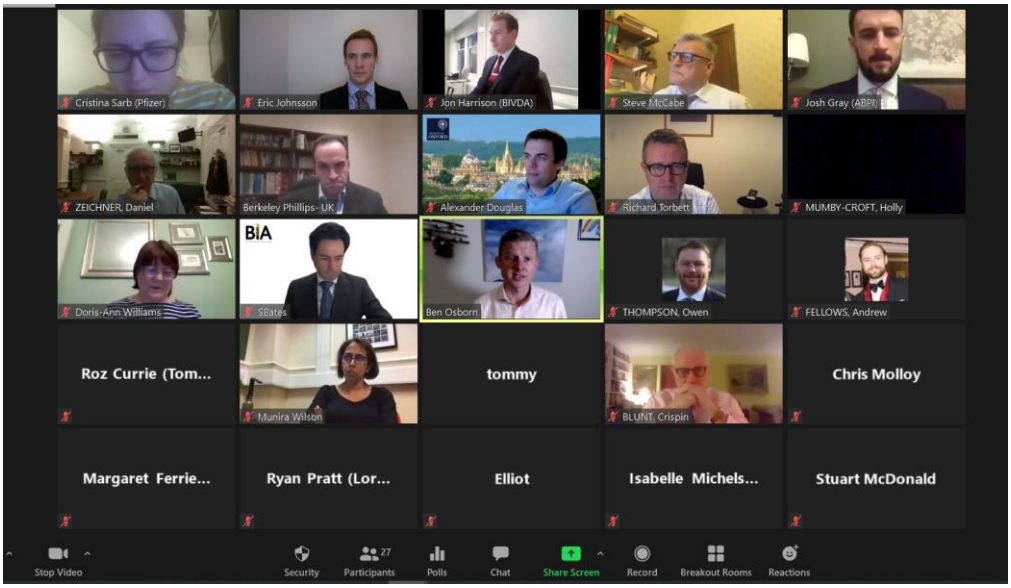
The **Life Sciences European Union Relationship Group** (EURG), an expert group of the LSC, met on 23 October and was co-chaired by Phil Thomson of GSK and DHSC Minister Lord Bethell, who was joined by Edward Argar (the Health Minister with responsibility for the future relationship with the EU) and BEIS Minister Nadhim Zahawi. The meeting was given an update on the negotiations as well as discussing preparations for the end of the Transition Period, the Northern Ireland Protocol and Continuity of Supply with June Raine and Jonathan Mogford of the MHRA speaking on regulation and guidance. The BIA takes part in the weekly government/industry meeting to take these agendas forward between EURG meetings.

The **Life Sciences Industrial Strategy Implementation Board** (LSISIB) met on 28 October, chaired by Lord Bethell and Professor Sir John Bell. The meeting reflected on the implications of the COVID-19 pandemic for the UK life sciences industry, received progress reports on the life sciences sector deals and an update on the life sciences strategy before concluding with a discussion of the Accelerating Detection of Disease programme.

The **Comprehensive Spending Review** has been postponed because of the pandemic, but a more limited **Spending Review** covering one year was carried out in November. For more information about the BIA's activities to influence the Government's priorities, see our Finance, Tax and Investment section below.

The **NICE Methods Review** (in which the BIA has been represented on the Steering Group and Task and Finish Groups) consultation went live in November and the BIA has submitted a response. The **All-Party Parliamentary Group (APPG) Life Sciences** held a meeting on the Methods Review and Access in October, this was followed by a well-attended meeting on COVID-19 on the day the Pfizer/BioNTech vaccinations started in the UK. On the same day, dubbed 'V Day' by Ministers, we also held our **annual MHRA-BIA bilateral meeting**.

The BIA is making representations on the **National Security and Investment Bill** which is currently before Parliament. The Bill introduces a mandatory notification regime for certain types of transactions in 17 key sectors, including Engineering Biology and AI. Read more about this on page 10.



The APPG for Life Sciences hosted a virtual briefing on COVID-19 vaccines and testing on the day the Pfizer/BioNTech vaccinations started in the UK.

EU and international relationships

UK secures trade deal with the EU

The final quarter of 2020 was a period of waiting to learn what the relationship between the EU and UK would be after 31 December. At the beginning of October, all eyes were on the deadline set by the Prime Minister for agreeing a deal, the EU Council Summit on 15 and 16 October. When this deadline was missed, the UK briefly broke off trade talks before agreeing the basis for negotiations to continue. From early November, each week there was an expectation that the following week would see the breakthrough. This cycle of anticipation and disappointment continued until Christmas Eve when a deal was announced.

Throughout this period, talks intensified and were increasingly insulated from outside influence. The BIA continued to push for mutual recognition of batch testing, the delayed implementation of new regulatory requirements in Northern Ireland, and to work with Government on potential medicinal products supply issues following the end of transition. MHRA guidance was published, the Department of Health and Social Care (DHSC) sent letters to medicinal products manufacturers, and the BIA ran four webinars providing updates on the talks and preparing for the end of transition. The webinars included expert speakers on new regulatory requirements and representatives from the Office for Life Sciences (OLS).

After the deal was announced on Christmas Eve, the BIA was able to quickly analyse the agreement and publish our analysis on our Brexit webpage. While a medicinal products annex was included, the annex did not include mutual recognition of batch testing.

Commenting on the deal, BIA CEO Steve Bates said: "Today's deal brings much desired certainty for our sector after four years of detailed negotiation. In his first remarks on the deal the Prime Minister mentioned positively the concept of 'mutual recognition' and stressed his desire for the UK to be a 'collaborative science superpower', 'able to set our own standards, innovate in the way we want, and to originate new frameworks for sectors like bioscience'. We look forward to working with the UK Government on this positive agenda, mindful of the fact that our sector seeks, and benefits from, innovative global standards and regulatory collaboration and co-operation. The UK bioscience sector has achieved global prominence in the last forty years and is set fair to thrive in the next forty."

With the transition period ending at 11pm on 31 December, the focus has now moved on to Britain's future. Many trade deals have been signed between the UK and other countries that replicate trading arrangements which had previously been agreed between those countries and the EU. Questions now focus on the unanticipated consequences and effects of the EU-UK trade deal, the potential for greater regulatory divergence from the EU, and what opportunities and requirements the UK's negotiations with other countries around the world may result in. To read more about what the deal means for medicines regulation, please see page 16. The BIA is committed to continue working with members, government and the wider life sciences sector to continue to understand and overcome issues, and to foster an environment that supports the UK's world-leading life science sector.

BIA leads international delegation to Geneva to highlight global industry's response to pandemic

In November, BIA CEO Steve Bates, in his role as Chair of the International Council of Biotechnology Associations (ICBA), led an international delegation of biotech industry associations in a series of meetings with diplomats at national missions in Geneva, senior WTO representatives, and the Director General of WIPO. The meetings focused on explaining the role of the global life sciences industry in the COVID-19 response, the important role of IP in biotech, and the need for the ICBA to engage more with the WHO.

Finance, tax and investment

New BIA analysis shows market-beating performance of UK quoted biotech

For the first time, the BIA has published an analysis of the share price performance and investor base of biotech companies quoted on the London stock markets. The report, published in the first week of 2021, revealed that the sector delivered market-beating returns in 2020 and attracted new investors looking for growth in a turbulent year.

The study, conducted for the BIA by Radnor Capital Partners, is targeted at generalist and retail investors both in the UK and internationally, with the aim of attracting more investment into the sector. It reveals 24 new American and 16 new European finance houses invested in the UK biotech sector for the first time in 2020, showing global recognition for UK innovation. 20 new UK institutional investors also entered the sector as other sectors of the economy were hit by the impacts of the pandemic.

Other key findings were:

- The combined share price of UK-quoted biotech companies was up 155% over the year
- Companies involved in diagnostics and COVID-19 treatments were the best performers (up 493% and 475%, respectively) and other sub-sectors including small molecule drug development (+286%), research tools and data (+153%) and cell and gene therapies (+149%) also performed exceptionally well
- £1,141m of fresh equity was raised by 51 individual companies, more than double the amount raised in 2018 and 2019 combined

BIA CEO Steve Bates said: “2020 was a year when biomedical innovation showed its true value, and it is great to see investors recognise that the UK has some of the best biotech companies in the world. Biotech and the broader healthcare sector are counter-cyclical in nature, meaning they can protect investors in periods of economic downturn and deliver asset growth due to the rapidly expanding global demand for better healthcare.

“With over £1 billion of fresh capital invested in 2020, biotech companies on the London Stock Exchange are ready for growth in 2021 and beyond, meaning life-changing treatments can be delivered to patients sooner and investors can see the many different ways in which our sector can create value.”

The report was covered by The Times and Yorkshire Post, amongst other media outlets.

BIA submits evidence to review of UK listing rules

The Government has launched a review of the UK’s listing rules to determine how more companies could be attracted to list on London stock markets. The review is being chaired by Lord Hill, former European Commissioner for Financial Stability, Financial Services and Capital Markets Union and the BIA has responded to his call for evidence. In our letter, we state that the life sciences sector attracts high levels of international investment but relatively little from UK institutional investors, and as a result, NASDAQ is the listing destination of choice for life science companies. We argue that this is not due to the UK's listing rules but NASDAQ's deep capital pool and experienced investors. The BIA hopes to meet Lord Hill in the coming months and will continue to engage in the review.

BIA reveals that squeezed government biomedical innovation budget puts future medicines at risk

Ahead of the Spending Review in November, the BIA revealed new data that showed that insufficient government funding is putting the UK's pipeline of future biomedical innovations at risk. Less than 4% of projects which were ranked as worthy of investment received funding from the Biomedical Catalyst in 2019/20, down from 31% in 2014/15.

The BIA obtained the data via a Freedom of Information (FOI) request to UKRI. The response showed that over the last three years, research project applications totalling a value of over £530 million have not been funded despite passing the quality threshold. The projects could have leveraged approximately £2.5 billion in private investment, estimates from IPSOS Mori suggest. The FOI data demonstrates that the budget for the Biomedical Catalyst is not sufficient to support the number of high-quality applications it is receiving. The story was covered in Research Professional and accompanied by a comment piece by Eric Johnsson, Policy and Public Affairs Manager at the BIA.

2020 Spending Review delivers for research while BIA continues to lobby for the Biomedical Catalyst

In the Spending Review delivered on 26 November, the Government continued to deliver on its promise to increase R&D investment with £15bn overall committed for 2021-22 and increases for UKRI core budgets over the next three years. However, Innovate UK received only a one-year flat budget settlement and there was no announcement on a Biomedical Catalyst competition for 2021, for which the BIA has campaigned. Read the BIA's full analysis of the 2020 Spending Review outcome on the BIA blog.

The BIA has been building a close relationship with the new UKRI CEO to ensure the needs of our sector are heard at the highest level within the funding body, which includes Innovate UK. BIA CEO Steve Bates met with Professor Dame Ottoline Leyser earlier this year prior to the Spending Review and BIA Chair Ruth McKernan and members of the BIA Board also met with Dame Ottoline in November.

Following the Spending Review, the BIA coordinated a letter on behalf of the life sciences sector to Business Minister Nadhim Zahawi MP, urging the Government to refill the Biomedical Catalyst in 2021 and increase its budget. The letter was signed by more than 100 senior leaders of life sciences companies and organisations.

Legislation published confirming exemptions to the R&D tax credit PAYE cap

The draft legislation setting out how the cap on SME R&D tax credit payments will operate, including the exemption to protect biotech companies for which the BIA has fought, was published in November. The initial proposals announced by the Government in 2018 would have severely impacted the sector. Since then, the BIA has campaigned publicly to raise concerns and worked with Treasury officials to develop a test within the legislation that will identify and exempt genuine companies that are not seeking to abuse the regime (see BIA submissions to HM Treasury here and here).

The draft legislation implements much of what the BIA proposed and should mean almost all genuine biotech companies should not have their tax credit payments capped. Since it was published, the BIA's Finance and Tax Advisory Committee has met with HMRC officials to discuss the finer points of the legislation and its implementation. The finalised legislation and cap regime will be confirmed in the coming months, potentially being announced alongside the Budget on 3 March, and will become effect from 1 April. For more information, contact Martin Turner on mturner@bioindustry.org.

BIA works with government to limit sector impact of the National Security and Investment Bill

In November, the Government introduced the National Security and Investment (NSI) Bill in Parliament. The Bill aims to provide the Government with updated powers to comprehensively scrutinise and intervene in certain types of transactions across the economy if they give rise to national security concerns.

In January, the BIA responded to the Government's consultation on the NSI Bill. In our response, we emphasised that the definitions of engineering biology and artificial intelligence (AI) will capture all companies within the UK life sciences sector and, as the vast majority of these companies have no implications for national security, the NSI regime would place undue burdens on hundreds of businesses in our sector and make the UK a less attractive place globally to start and build life sciences companies. We also argued that the implementation of the regime must not deter foreign investment in the UK life sciences sector, that it is critical that Department for Business, Energy and Industrial Strategy (BEIS) is sufficiently staffed and has the level of expertise required to respond to notifications appropriately and quickly, and that the Government should provide further detail on technologies that it considers to be a national security concern. The BIA's full submission is available on our website.

The BIA has also met with the civil servants leading on the NSI regime to discuss how the regime could be adjusted to avoid negatively impacting investors, SMEs and large companies that are active, or looking to become active, in the UK's life sciences sector. We will work to ensure the Government understands how the regime will affect the sector and keep our members informed as the Bill goes through Parliament.

BIA and British Business Bank collaborate on analysis of UK biotech VC fund returns

In 2020, the BIA worked with the British Business Bank to collect and analyse data on the performance of UK-based life sciences venture capital funds to address the lack of publicly available information on which investors can make informed investment decisions. The report, published in November, was the most comprehensive analysis of UK life science venture capital performance to date and it revealed that life science investments have performed equally or better than those in other sectors. However, while the findings were positive, the dataset is too young and shallow to be conclusive; it will become richer and more accurate as the cohort of venture funds grows and matures. The BIA will continue to work with the British Business Bank in future years. Read the BIA's analysis and the full report on the BIA website.

BIA urges government to ensure pension funds are encouraged to invest in British innovation

The BIA has written to Pensions Minister Guy Opperman MP in response to the Department for Work and Pensions consultation on improving outcomes for members of defined contribution schemes. We agreed with the consultation's conclusion that the pervasive focus on low fees within the pensions industry acts as an undesirable barrier to investing in illiquid assets, most significantly venture capital due to its costs. The charge cap adds to this impression so the Government's clarification that low fees does not equal good value, along with changes to the calculation of the cap to permit investment strategies subject to higher fees, is welcome. However, we warned the Minister that this would not result in a significant change of behaviour within the risk-averse pensions industry and that more active measures may be required to ensure pensions savers' money is invested into growth opportunities rather than low-risk, low-return securities. We instead recommended that the Government looks to create its own collective investment vehicle to enable pensions to co-invest in venture capital.

Strategic technologies and areas of scientific focus

BIA urges DEFRA to re-evaluate UK guidance on access and benefit sharing

As the UK's transition period with the EU ended on 31 December 2020 and was replaced by the EU-UK Trade and Cooperation Agreement, the EU Access and Benefit Sharing (ABS) Regulation has now been carried over into UK law. The BIA expects that the UK will develop its own guidance document to the UK regulation, which is likely to be largely based on the EU guidance document. However, as the EU guidance document is highly problematic for innovative organisations, the BIA has continued to urge the Government to re-evaluate the UK guidance document to ensure that it sets out unambiguous and helpful guidance that recognises how life sciences R&D is conducted.

People, skills and talent

Cell and Gene Therapy Catapult launches the Advanced Therapies Skills Training Network

This quarter, the BIA welcomed the launch of the Advanced Therapies Skills Training Network (ATSTN) as a national initiative to drive growth across the advanced therapies and vaccine manufacturing industry. The Network is driven by industry, coordinated by the Cell and Gene Therapy Catapult and backed by £4.7m investment from the Department for Business, Energy & Industrial Strategy (BEIS) and Innovate UK. The Network consists of an Online Training Platform (OTP) which showcases existing learning programmes, a Careers Converter for people to identify how they can enter the life sciences industry from other sectors, and a network of National Training Centres (NTC).

The ATSTN-OTP and the Careers Convertor were launched at the BIA's 2020 bioProcessUK Conference in a digital skills workshop opened by Amanda Solloway MP, Minister for Science, Research and Innovation. Industry licenses are now available on request from the ATSTN website.

In late December, the National Horizons Centre, RoslinCT and the University of Birmingham were announced as preferred bidders to deliver high impact physical and digitally delivered training courses as part of the ATSTN-NTC. The three centres bring with them complementary capabilities and a vast wealth of experience across GMP/GxP, manufacturing and bioprocessing. Their expertise within virtual reality training will prove instrumental for driving the successful development of cell and gene therapy as well as vaccine manufacturing staff across the UK, through the delivery of these industry-leading training courses.

Amanda Solloway MP, Minister for Science, Research and Innovation, launches the Advanced Therapies Skills Training Network at bioProcess UK.

Intellectual property and technology transfer

Allow AI-derived inventions to be patentable, argues the BIA

The BIA has responded to the UK Intellectual Property Office's call for views on AI and IP outlining the growing importance of AI in life sciences and the need to offer the same protection to inventions generated through the use of AI as is offered to human-generated inventions. Patents currently require a named human inventor, which is increasingly becoming an ambiguous concept as AI technology grows in power and capability. The BIA therefore argued that inventions will increasingly be derived solely from the work of AI rather than human thinking, and that these advances are of equal value and importance, meaning it is critical that the companies that own the AI technology are able to protect them in the same way they can for those derived from their employees. The submission was written by the BIA's IP Advisory Committee, which has established a sub-committee to monitor and further influence policy in this field.

Post-Brexit SPC regime becomes law

Secondary legislation establishing a new Supplementary Protection Certificate (SPC) regime to address the separate medicines regulatory regimes in Great Britain and Northern Ireland was passed by Parliament in December and came into effect following the UK-EU trade agreement. The new regime allows for UK SPCs that cover only the region (Great Britain or Northern Ireland) to which a marketing authorisation applies, which is separately approved by either the UK's MHRA for Great Britain or the EMA for Northern Ireland. The BIA's IP Advisory Committee provided feedback on the policy design and legislation prior to its Parliamentary approval. The Government has published business guidance on the new regime.

Pre-clinical and clinical research

BIA Antibody Taskforce identifies novel COVID-19 antibody therapy candidates

In October, the UK BIA Antibody Taskforce, a leading UK consortium developing antibodies for treating COVID-19 reached a major milestone - the identification of differentiated antibody combinations that will be taken forward for further development as an antibody 'cocktail'. The Taskforce developed an accelerated and rigorous multifaceted approach to create a pool of over 600 novel candidates and identified a set of antibodies with the greatest potential.

Dr Jane Osbourn OBE, CSO at Alchemab and leader of the Taskforce, said: "We have accelerated the standard timelines for antibody discovery, taking seven months rather than the industry standard 18 months, establishing a pathway that can be applied to future pandemics. We believe that the most effective tool against COVID-19 will most likely be a defined mixture of two to three antibodies - so the effectiveness of different combinations must also be assessed."

These candidate antibodies are the first to be selected for the next stage of development, following assessment by collaborators for potency. Assessment of their efficacy as a cocktail is on-going, however, early indications are showing a potential competitively potent cocktail that is differentiated from other products currently being investigated in clinic. The candidates are poised to move into the next phase of development where further screening and safety testing will need to be performed.

Steve Bates said: "This ground-breaking Taskforce has brought together UK-based industry experts who share a joint commitment to help fight the COVID-19 pandemic. Contributing their expertise and specialist resources, the team has worked tirelessly to identify the most promising antibodies with the potential to positively impact treatment of those affected by this devastating virus. The next stage will involve securing external funding to facilitate the further development and manufacture of the candidates."

The next phase of the project will involve manufacturing of the selected antibody cocktails to support initial clinical trials in 2021, to provide essential safety and efficacy data.

BIA Antibody Taskforce is a world-leading consortium of biotech companies, charities and academia experts based across the UK who share a joint commitment to help fight the COVID-19 pandemic.

Manufacturing

BIA hosts 17th annual bioProcessUK Conference

The 17th annual bioProcessUK conference, which took place virtually in December, shone a light on the work our bioprocessing community has done in response to COVID-19, particularly in vaccine manufacturing. We heard from speakers who have been involved first-hand with vaccines and therapeutics development, including Kate Bingham, Chair of the UK Vaccines Taskforce. Other sessions included a discussion on digital skills, a workshop on how arthritis therapies have evolved over the years, a Dragon's Den style technology showcase and a panel which considered how industry 4.0 is being applied in our sector. Delegates also enjoyed virtual networking and beer tasting at the BioProcess Arms.

Kate Bingham, Chair of the Vaccines Taskforce, gives an update at bioProcess UK.

The event was also an opportunity to celebrate outstanding achievements: congratulations to Steve Bagshaw, Chairman of FUJIFILM Diosynth Biotechnologies, and the BIA COVID-19 Vaccines Manufacturing Taskforce, who were awarded the prestigious Peter Dunnill Award and Richard Wilson Impact Award respectively. These worthy recipients have been recognised for their work and contributions to the bioprocessing and biologics manufacturing sector. You can find out more about the awards on our website.

BIA's COVID-19 Vaccine Manufacturing Expert Advisory Group continue to advise government

The BIA's manufacturing community has been at the heart of efforts to understand and overcome the challenges of the manufacturing and scale-up of a successful COVID-19 vaccine in the UK. Industry and academics from across the UK have contributed their expertise and resources to find solutions to this monumental challenge by supporting both the Oxford University Jenner Institute's ChAdOx and Imperial College London's RNA vaccine. As these projects moved from advice to multi-million-pound contracts, there was a natural stepping-off point for the BIA as the point of delivery coordination. That moment arrived in July, where we handed over the delivery baton to the UK Government Vaccines Taskforce. The experts and manufacturing capacity organised via our initial Taskforce continue to deliver their vaccines work directly to the Government. The story of the BIA COVID-19 Vaccine Manufacturing Taskforce has been documented in this case study and was shared at bioProcessUK.

The BIA retains a strategic advisory function ready to help through an Expert Advisory Group, chaired by Ian McCubbin OBE, but is not responsible for overseeing the programme management of what has become a series of key government deliverables. This Expert Advisory Group has contributed to the recently published UK Vaccine Taskforce 2020 Achievements and Future Strategy End of year report and continues to support with the manufacture of emerging vaccines and antibodies against COVID-19.

Medicines Regulation

BIA attends EMA Industry Stakeholders meeting on the UK withdrawal from the EU

On 30 November, the BIA participated together with EuropaBio in an EMA Industry Stakeholders meeting to discuss the practical aspects of the implementation of the Northern Ireland Protocol regarding centrally authorised medicinal products after the end of the Brexit transition period. During the meeting, which was also attended by EU member states representatives, the European Commission clarified that any EU-UK future relationship would be very different from those with EU/EEA countries and from other third countries. The meeting report and presentations are available on the EMA website here.

Detailed additional practical guidance as Questions and answers to stakeholders on the implementation of the Protocol on Ireland / Northern Ireland was published by EMA in December. This includes the consequential changes to Article 57 database, fee calculation, safety reporting into EudraVigilance and access to EudraVigilance data, other IT tools and systems, GMP and manufacturing, SME incentives and orphan designation sponsors, marketing status and dossier submission.

BIA publishes blog on what the UK-EU agreement means for medicines regulation

The UK and EU negotiators reached agreement on the future relationship just days before the end of the Brexit transition period, ending UK's participation in the EU's Single Market and Customs Union. The BIA has published a blog on what the UK-EU Trade and Cooperation Agreement means for medicines regulation, which is available here.

The blog also updates on the implementation of the Northern Ireland Protocol which forms part of the Withdrawal Agreement. From 1 January 2021, EU pharmaceutical law applies to the UK in respect of Northern Ireland only. The European Commission published a notice on 22 December 2020 which provides practical details on the 1-year phased process for implementing certain aspects of medicines regulation in Northern Ireland. Companies need more time to prepare in relation to batch testing, importation and Falsified Medicines Directive requirements to ensure the continuity of supply and patients getting the medicines they need.

The MHRA issued updated guidance to industry to follow for a period of two years from 1 January 2021. The BIA has been a key consultee working closely with the MHRA to develop guidance for the end of transition period.

BIA welcomes the MHRA Innovative Licensing and Access Pathway

On 1 January, the MHRA launched a new medicines approval pathway in the UK – the Innovative Licensing and Access Pathway, which was first announced at the joint BIA-MHRA Conference last September. The pathway provides a single integrated platform for collaborative working between the MHRA, NICE, the Scottish Medicines Consortium, NHS England and NHS Improvement and the medicine developer to improve efficiency and to accelerate the time to market. The BIA supports a joined-up life sciences ecosystem which would make the UK more attractive for research and development of innovative medicines to the benefit of patients.

A new innovative medicine designation, the 'Innovation Passport', acts as the gateway to entry into the pathway and is awarded to new medicines undergoing development, including advanced therapy medicinal products, medicines for rare diseases, new indications and repurposed medicines. Further information about the ILAP and how to apply for an Innovation Passport is available on the MHRA website.

BIA supports UK international cooperation with Australia, Canada, Singapore, Switzerland and US regulators

The MHRA is building collaboration with global regulatory authorities and is now part of the Access Consortium alongside the Therapeutic Goods Administration of Australia, Health Canada, Health Sciences Authority of Singapore and Swissmedic. The Consortium aims to bring timely access to high quality, safe and effective medicines to a larger population.

The MHRA commenced work-sharing applications with the Access Consortium partners from 1 January 2021. The Access Consortium published its Terms of Reference and developed two authorisation procedures: the Access New Active Substance Work Sharing Initiative and the Access Generic Medicines Work Sharing Initiative.

Additionally, the MHRA joined Project Orbis, a programme coordinated by the US FDA to review and approve promising cancer treatments, involving the regulatory authorities of Australia, Canada, Singapore, Switzerland, Brazil, as well as the UK. For more information, see the Guidance on Project Orbis. The BIA welcomes the MHRA's involvement in this regulatory path because as nearly all innovative cancer medicines are launched in the US, it could become a key route for the UK to remain an early and priority market for global launches after the end of the Brexit transition period.

BIA view on Statutory Instrument for the rollout of COVID-19 vaccines

The BIA published a blog post to explain the position the BIA took during the expeditated consultation on changes to the Human Medicine Regulations to support the effective rollout of COVID-19 vaccines and the upscaling of flu vaccination programme in the UK.

The BIA is supportive of the Government's general approach, given the impact of the pandemic on public health and the economic wellbeing of the nation, and the need to deal with the biggest threat the UK has faced in peacetime. Our feedback is focused on specific areas of concern or where greater clarity may be required. The blog is available on our website.

BIA responds to MHRA consultations on real world evidence guidance and guidance on licensing of biosimilar medicines

In December, the BIA, with the input of member companies on the BIA Regulatory Affairs advisory Committee, developed a consolidated industry response to the consultation on the MHRA draft guidance on randomised controlled trials generating real-world evidence to support regulatory decisions. This guidance is intended to be the first in a series of guidance documents addressing issues around using real-world evidence in support of a regulatory submission.

In November, the BIA, in collaboration with the ABPI and IFPMA, submitted a joint response to the MHRA consultation on the proposed guidance for the UK licensing of similar biological medicinal products at the end of the transition period from January 2021. The MHRA guidance contains some revisions to the EMA's Committee for Medicinal Products for Human Use (CHMP) guidance documents, taking into account the scientific and regulatory experience gained since the first biosimilar product was authorised in 2006. These revisions cover details for UK reference products, in vivo studies in animals not being required and changes in the requirement for a comparative efficacy trial.

Access to medicines

BIA's Rare Disease Industry Group and PwC launch report on a new framework for rare and ultra-rare disease

In November, the BIA's Rare Disease Industry Group (RDIG) published *A rare chance for reform* with PwC. Based on a set of specific recommendations, the report sets out a new way forward for evaluating medicines for rare and ultra-rare diseases in England. The report identifies key areas where reform would remove barriers and ensure access to rare disease medicines. Our recommendations include:

- Accelerate access through a conditional access period
- Address systemic issues to build a strong environment for access to orphan and ultra-orphan medicines
- Update the evaluation framework to better account for the unique challenges of rare and ultra-rare diseases
- Evaluate orphan medicines and ultra-orphan medicines through a single rare disease pathway
- Assess empirically based Incremental Cost-Effectiveness Ratio (ICER) thresholds on a sliding scale
- Continue to create a supportive atmosphere for patient groups

RDIG has agreed to an ambitious programme of work which will see the group's persistent advocacy efforts carry on into 2021 and the policy recommendations of the report will form the evidence base for the group's advocacy work.

The BIA and PwC published a report on rare and ultra-rare disease in November.

BIA responds to the first consultation of the NICE Methods Review

In December, the BIA responded to the first consultation on the case for change to NICE's methods. This is the culmination of months of work drawing on the expertise in our Rare Disease Industry Group (RDIG), working with industry partners and developing our position (see the report *A rare chance for reform,* above). The NICE Methods Review provides a unique opportunity to shape the principles that will frame the medicines access landscape for many years in the future.

We had previously secured member representation on several Task and Finish groups which looked in detail at the key areas for review. A significant part of our work throughout 2020 included supporting engagement with these Task and Finish groups. The reports from all of the groups can be found here. In addition to the Task and Finish Groups, we also secured representation on the Methods Review Working Group which coordinated the development of NICE's proposals. This work resulted in effective engagement between NICE, industry, and patient groups, which has led to an open and effective process.

The review will now enter a new phase where NICE will review consultation responses, while industry will continue to engage with NICE on two new working groups which will further explore challenging technologies and NICE decision-making. Two further consultations will take place in 2021 and we look forward to responding to these.

BIA policies highlighted in the House of Lords

As the House of Lords examined the Medicines and Medical Devices Bill, Peers have been involved in constructive debates on rapid adoption of innovative medicines and patients' access to medicines for rare diseases. The BIA briefed relevant Peers ahead of these debates and we were encouraged to see our policies being raised in a debate on the Innovative Medicines Fund (IMF) in the Grand Committee. The BIA hopes the IMF will facilitate access to innovative rare and ultra-rare disease medicines, where the level of unmet need is high. It was especially positive to see Lord O'Shaughnessy mentioning the BIA and agreeing with us that an ambitious definition of 'innovation' is required for the fund to improve access.

More recently, during the Bill's report stage, we were encouraged to see Lord Hunt of Kings Heath and Lord Lansley raise the issues of patients' access to medicines for rare diseases, holistic value-based pricing, and the introduction of differential discounting rates for costs and health effects (see previous section). These are policies we support and advocate for in *A rare chance for reform* and in our response to the NICE Methods Review consultation.

BIA reacts to launch of new Rare Diseases Framework

In January, the Government published its long-awaited Rare Diseases Framework, which provides a promising vision for the future. It replaces the 2013 UK Strategy for Rare Diseases and sets out common priorities for the UK's four nations to ensure the lives of people living with rare diseases continue to improve.

The Framework is a positive step forward in areas such as patients' access to treatments and the use of genomics in diagnosis, but we will see the final detail in the nation-specific action plans, which are due to be launched later this year. We look forward to engaging with this process to ensure people affected by rare and ultra-rare diseases can access the life-changing treatments they need. For more detail, please see our blog.

For more information on the BIA's activities in policy and regulatory affairs, or to share feedback on this report, please contact Eric Johnsson, Policy and Public Affairs Manager, 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, Senior Membership and Business Development Manager, on 0207 630 2194 or mmcgivern@bioindustry.org

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