Charging for technology appraisal and highly specialised technology recommendations and miscellaneous amendments to NICE legislation – BIA's response

September 2018

UK BioIndustry Association

Introduction and overview

The BioIndustry Association (BIA) is the trade association for innovative life sciences in the UK. Our goal is to secure the UK's position as a global hub and as the best location for innovative research and commercialisation, enabling our world-leading research base to deliver healthcare solutions that can truly make a difference to people's lives. Our membership includes large, multinational pharmaceutical and biotech companies as well as SMEs. Collectively, BIA members are responsible for over 90% of biotech medicines currently in clinical development in the UK.

The BIA welcomes the opportunity to respond to this consultation. NICE Technology Appraisals are an extremely important part of the regulation and scrutiny of medicines. Industry understands that NICE needs adequate funding and resources to allow it to evolve its processes and methods to keep up with new technologies, such as advanced therapies and precision medicines, now coming to market. Due to their novel methods of administration, potential long-term therapeutic benefits and often small patient populations these technologies will present NICE with new challenges. The BIA recognises the value of ensuring NICE is sustainable and continues to be a global leader in health economics and medicine assessment, particularly as the science evolves, and we are keen to play our part in achieving this. However, we have some concerns about the timing of this consultation and the potential impact of the proposals on the future prosperity of the UK life science sector, which delivers significant benefits for UK health and wealth.

Our submission focuses solely on the introduction of charges for NICE Technology Appraisals and builds on our response to the previous consultation on this issue, led by NICE in September 2016.

Key points

- The BIA is concerned about the message this consultation, and other proposed changes affecting UK life sciences, are sending to the international life science community about the UK as a location to research, develop and launch innovative medicines.
- Brexit is creating a great deal of uncertainty for UK life science companies and depending on the outcome of the negotiations, the UK's exit from the EU could pose a number of risks for the success and sustainability of the UK's life science sector. As the UK prepares to leave the EU, the delivery of an internationally-competitive industrial environment for the bioscience and life-sciences sector is more important than ever.
- In addition, the sector is currently being consulted on revisions to the Statutory Pricing Scheme and, via the ABPI, is negotiating a new Pharmaceutical Pricing Regulation Scheme. While industry is being asked

to respond to these activities separately they will coalesce in terms of timing and their impact on the sector. Furthermore, industry is yet to see the full impact of changes introduced to NICE Technology Appraisals in April 2017, including the Budget Impact Threshold and the introduction of a cost-per-QALY threshold for Highly Specialised Technologies (HSTs), and earlier this year to increase capacity. The BIA believes that the potential negative impacts of the proposals set out in this consultation will be compounded by broader developments affecting the sector and it calls on the government to take this in to consideration when deciding next steps.

- Following the EU referendum, the government has consistently stressed the strategic importance of the UK biopharmaceutical sector to the UK's future prosperity. The changes proposed in this consultation, and the broader developments affecting UK life science companies, go against this. Government needs to be consistent and transparent about its ambitions for the sector.
- If government decides to go ahead with the proposed changes, their implementation must be accompanied by clear plans for improving NICE processes and metrics to evaluate success, both of which should be co-designed with industry.
- The BIA welcomes the consideration given to the impacts the proposed changes will have on SMEs and notes the addition of measures to mitigate these impacts following the previous consultation on this issue in September 2016. The BIA believes however, that despite these measures the introduction of charges for NICE Technology Appraisals will still have a disproportionate effect on SMEs, particularly those that are pre-revenue, and if introduced, could disincentivise these companies from launching their products in the UK.
- The BIA also believes that the proposed changes will have a negative impact on patient access to medicines and create inequalities across the UK. This will disproportionately affect patients with rare and ultra-rare conditions.

Impact on Investment and the Future Success of the UK Life Science Sector

The BIA is concerned about the message this consultation, and other proposed changes affecting UK life sciences, are sending to the international life science community about the UK as a location to research, develop and launch innovate medicines. Bioscience companies, even if they are based in the UK, make their commercial decisions from a global perspective. With the UK's exit from the EU on the horizon, now is a critical time for demonstrating to global markets and companies that the UK remains an excellent location to invest and develop medicines. The BIA believes that introducing charges for NICE Technology Appraisals will send a negative signal globally and impact investment in the UK and decisions about the location of launching products.

Brexit is creating a great deal of uncertainty for UK life science companies and depending on the outcome of the negotiations, the UK's exit from the EU could pose a number of risks for the success and sustainability of the UK's life science sector. For instance, recently published government guidance has now made it clear that a 'no deal' Brexit would mean a significant increase in replicative bureaucratic red tape for developers of innovative medicines that choose to sell their products in the UK. Even if the UK negotiates a deal with the EU, operating costs for UK companies will potentially increase. Additional regulatory processes and costs for a market worth less than 3% of the world's value will most likely

diminish the UK's attractiveness for life science businesses and may mean that NHS patients get access to new therapies later than other countries in Europe. Introducing charges for NICE Technology Appraisals at the same time would only serve to exacerbate these challenges. Given its breadth of expertise and global reputation, NICE could be a draw for bioscience companies considering launching their products in the UK post-Brexit, particularly if it can establish itself as a leader in the assessment of the new era of more personalised treatments. However, this is unlikely to be the case if charges for NICE Technology Appraisals are introduced.

In addition, the life science industry is currently being consulted on revisions to the Statutory Pricing Scheme and, via the ABPI, is negotiating a new Pharmaceutical Pricing Regulation Scheme. While industry is being asked to respond to these activities separately they will coalesce in terms of timing and their impact on the sector. Industry is also yet to see the full impact of changes introduced to NICE Technology Appraisals in April 2017, including the Budget Impact Threshold and the introduction of a cost-per-QALY threshold for Highly Specialised Technologies (HSTs), and earlier this year to increase capacity.

Following the EU referendum, the government has consistently stressed the strategic importance of the UK biopharmaceutical sector to the UK's future prosperity however, the changes proposed in this consultation, and broader developments affecting UK life science companies, go against this. The BIA believes that in conjunction with broader developments, the proposals set out in this consultation are likely to result in a poor commercial environment which, when further exacerbated by the complications of Brexit, will reduce the attractiveness of the UK as a place for bioscience companies to do business. The government should consider the collective impact of broader developments, including Brexit, on the UK's life science ecosystem when deciding how to take the proposals set out in this consultation forward and ensure it is consistent and transparent about its ambitions for the sector.

If government decides to go ahead with the proposed changes, their implementation must be accompanied by clear plans for improving NICE processes and metrics to evaluate success. These should be co-designed with industry and should include:

- Increased flexibility for applicant companies e.g. companies should be able to choose what type of appraisal they would like to progress with assuming their product meets the eligible criteria
- Improvements in appraisal timelines
- A greater focus on the implementation of NICE recommendations
- The evolution of NICE processes to keep pace with cutting-edge science and innovation e.g. gene therapies, cell therapies and personalised treatments, which will naturally present with greater uncertainty in their data sets.

Impact on SMEs

The UK is home to a vibrant SME bioscience industry ecosystem. 79% of the UK's 2000 bioscience companies are SMEs. The BIA therefore, welcomes the consideration given to the impacts the proposed changes will have on SMEs and notes the addition of measures to mitigate these impacts following the previous consultation on this issue in September 2016. The BIA believes however, that despite these measures, the introduction of charges for NICE Technology Appraisals will still have a disproportionate

effect on SMEs, particularly those that are pre-revenue, and if introduced, would disincentivise these companies from launching their products in the UK. Biopharmaceutical companies already encounter significant costs when preparing their HTA submissions and this comes with additional risks for SMEs that are unlikely to have alternative products on the market that can bring in extra revenue and cover potential losses. A negative NICE appraisal can also have a detrimental impact on a company's global reputation, significantly affecting their chances of securing market access outside the UK. This again poses higher risks for smaller companies with few or no products already bringing in revenue. Consequently, the introduction of charges for NICE Technology Appraisals may prohibit SMEs from seeking NICE approval, and subsequently access to their medicines for NHS patients.

In addition, whilst SMEs are more likely to have their headquarters in the UK and undertake their R&D in the UK, they still make their commercial decisions from a global perspective. If the introduction of charges for NICE Technology Appraisals is not prohibitive for SMEs it will likely encourage them to seek access in alternative global markets as a priority in order to secure a revenue stream that can mitigate against losses encountered when seeking NICE approval. As a result, the introduction of charging is likely to result in UK patients either not benefitting from or experiencing delayed access to medicines developed by the UK grown life sciences ecosystem, which is in part funded by UK citizens.

Impact on Patients

As indicated previously, the BIA believes that the introduction of charges for NICE Technology Appraisals will either prohibit or delay patient access to medicines in England. This could result in inequalities across the UK as medicines may be made available to patients in Wales and Scotland, where charges for technology appraisals have not been introduced, before receiving approval for use on the NHS in England.

This will disproportionately affect patients with very rare conditions, thus undermining the government's commitment in the UK Rare Disease Strategy to ensure equity of access across all four UK nations. With the introduction of the charges proposed, it is possible that the cost of securing access for medicines with small patient populations will surpass the return on investment. This is even more likely given the introduction of tighter cost-effectiveness criteria to the NICE Highly Specialised Technology (HST) programme in 2017.

In contrast, the Scottish Government recently introduced a new pathway for ultra-orphan medicines (those medicines that treat fewer than 1 in 50,000 people), which allows patients to access these medicines for at least three years while data on their effectiveness is gathered. The introduction of the charges for the NICE HST programme would further diminish the appeal of launching ultra-orphan medicines in England compared to elsewhere. While other nations, inside and outside the UK, are putting measures in place to facilitate faster, fairer access to medicines for very rare and very rare conditions, England is creating additional barriers that are a best slowing down, and at worst prohibiting access to life-transforming treatments for patients with unmet need.

Alternative proposals

Given our previous comments about broader developments currently affecting UK life sciences, we believe that it is not appropriate to be consulting on the introduction of charges for technology appraisals at this time and we urge the government to delay consideration of these proposals until there is further clarity around the impact Brexit will have on the sector.

We do however appreciate that there is a pressing need to address NICE's sustainability and its ability to evolve and adapt to meet the challenges presented by the current pipeline of medicines. We are aware that in its response to this consultation, the ABPI has put forward an alternative proposal, which we believe is worthy of further exploration if the government decides to go ahead with the introduction of a financial contribution from industry to support NICE at this time.

ABPI suggests that industry's contribution should be collected via an aggregated payment mechanism built into the annual payment percentage for both the voluntary and statutory pricing scheme. It proposes that the final mechanism is agreed and worked up as part of the current PPRS negotiations, which ABPI lead on for industry. The BIA understands that there is significant support for this proposal among ABPI members, many of whom are also BIA members. We therefore support ABPI's proposal in principle but further consultation on the detail of the payment mechanism would be required to ensure that it meets the needs of the broader UK life science sector, including SMEs.

For additional information or clarification on any of the points raised please contact Rachael Stewart, Policy and Public Affairs Manager at rstewart@bioindustry.org or on 0207 630 2187.