Results of BIA survey: UK R&D incentives for orphan medicines in no-deal Brexit

November 2018

UK BioIndustry Association

Introduction

The BioIndustry Association (BIA) is the trade association for innovative bioscience companies in the UK. The BIA represents over 350 members, including emerging and more established life science companies; pharmaceutical companies; academic, research and philanthropic organisations; and service providers to the sector.

The BIA submission to the MHRA consultation on EU Exit no-deal contingency legislation highlighted our members' concerns regarding the UK proposals on orphan medicine designation. The full consultation response can be found here.

No measures were proposed in the consultation to replace the R&D incentives for innovative biotech companies currently provided in EU legislation. This would make the UK market less attractive for these companies wanting to launch orphan medicines.

Specifically, the MHRA is not proposing to duplicate the EU pre-approval orphan designation as "this will be available at EU level and that a separate UK only designation is unlikely to further incentivise industry to warrant the investment required to resource a separate system". Such an approach would remove R&D incentives to support the development of orphan medicines for the treatment of rare conditions. Instead the MHRA has proposed to retain this incentive – 10 years market exclusivity awarded at the time of marketing authorisation after evaluation of compliance with orphan criteria.

Companies which obtain orphan designation for their product in the EU can benefit from a range of incentives including fee reductions for scientific advice/protocol assistance and further incentives for SMEs.

At the request of the DHSC and MHRA the BIA carried out a survey among its member companies targeting UK-based SMEs and start-up companies involved in the discovery and development of new and innovative medicines. We asked members to feedback to us their view on the proposals and impact on their development strategy in the absence of any UK-specific support/incentives for orphan products, and what solutions they might suggest to UK government.

We welcome the opportunity to submit these comments and look forward to continued engagement with the MHRA and DHSC to address this area of concern as they work towards the final statutory instruments.

BIA recommendation

The outcome of the BIA survey shows the need for the UK to offer R&D incentives for orphan medicines in early clinical development and not only downstream marketing authorisation incentives in case of a no-deal Brexit.

With the aim of promoting and supporting innovation and the UK life sciences sector, the BIA calls on government to grant funding to the MHRA enabling the Agency to offer pre-market incentives for orphan medicines to SMEs in line with other European regulatory agencies. Such funding should be, in budget terms, ring fenced for a sufficient period of time.

BIA survey: key findings

Our research elicited responses from twelve companies:

- eleven SMEs and emerging companies, based in England, Wales and Scotland, which are involved in the discovery and development of new products
- a small regulatory affairs and product development consultancy company

Metrics

The UK-based SMEs and start-up companies indicated that they have products in clinical development with an orphan designation or are planning to apply for orphan designation for a drug candidate next year. In general, the orphan designation was granted in the EU, while some companies were seeking or had already received orphan designation for their product in both the EU and US.

The orphan designated products are intended for example for the treatment of rare and chronic endocrine conditions, cancer, respiratory infections in cystic fibrosis patients, etc.

The proposed system would limit flexibility for UK-based SMEs developing orphan medicines

All respondents emphasised the negative implications of the MHRA proposals for UK-based SMEs and start-up companies. Orphan designation is an important element of their development strategy as it is an important value driver for a small biotech company. Indeed, obtaining an orphan designation is a key step in gaining financial support from investors and developing the product to meet MHRA requirements.

Some member companies were of the view that they “want orphan designation from the get-go to benefit from free scientific advice from the UK regulators”.

Other developers of orphan medicines indicated that they would continue to seek regulatory advice at EU level during development and will establish a presence in the EU/EEA in order to continue to benefit from the EU incentives and fee waivers for SMEs.

Member companies indicated that it will be increasingly challenging to justify placing clinical trials in the UK in the absence of early engagement with UK regulators. One respondent said, “why would we do any clinical trials here”, adding they would have to conduct trials in a number of countries with an orphan designated product.

The lack of any UK support/incentives before the marketing authorisation stage would send the wrong message to UK and non UK-based companies developing orphan medicinal products. In the absence of such support, there is a risk that companies will not seek any advice from the MHRA during development and focus their attention on securing regulatory advice from the larger markets (i.e. EU and US). In this circumstance, the data submitted in the application dossier for marketing authorisation may not be acceptable to the MHRA, and there could be delays to the approval of innovative orphan medicines in the UK.

Finally, the assessment of compliance with orphan criteria at the time of UK marketing authorisation rather than before marketing authorisation introduces some uncertainty that may reflect adversely for the UK when applicants plan their global launch strategy. It is worth noting that SMEs are more likely to partner after Phase II trials and may not take orphan medicines to market.

Solutions suggested to UK government to address this situation

Incentivising the development and marketing of medicines for rare diseases has been very successful in the EU, and similar incentives should be made available for UK-based SMEs to maintain a vibrant life science sector.

Therefore, our member companies unanimously proposed recognition by the MHRA of the EMA opinion on the orphan designation application. It is most unlikely that products with an EU orphan designation will not also be orphan conditions in the UK. If that was a concern, the MHRA could ask applicants for scientific advice to provide brief data that shows the prevalence of the condition in the UK is similar to that in the EU. This would seem like a sensible approach in line with the concept of targeted assessment that MHRA is proposing for marketing authorisation applications for new medicines which have been submitted to the EMA through the centralised procedure.

It is therefore important to put in place a UK R&D incentives system to encourage SMEs and start-up companies to engage early with the MHRA and seek scientific advice/protocol assistance during the development of orphan medicines in order to ensure that UK patients are not denied access to or suffer unnecessary delays in access to such medicines.

For further Information please contact Dr Christiane Abouzeid, Head of Regulatory Affairs, cabouzeid@bioindustry.org