A new dawn in regulation and access

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Getting it right from the start is the preferred choice for any business and bringing medicines to market is no exception. As of 1 January 2021, the UK implemented a new regulatory and access pathway with a value proposition of end-to-end collaboration between the medicine developers, the regulatory agency, the health technology assessment (HTA) agencies, the payer, patients, and other partners. The key aim is to reduce the time to market for innovative medicines.

Following the departure from the European regulatory system, the UK government as part of its vision for life sciences has taken the opportunity to create an innovative regulatory framework, which is aimed at supporting and enhancing innovation and to accelerate routes to market[1]. Led by the MHRA, the Innovative Licensing and Access Pathway (ILAP) offers a new approach for companies who wish to reduce the time to market and increase patient access for innovative medicines. The pathway is open to developers from an early stage, even for products in preclinical phases of development.

The ILAP brings together the MHRA, NICE, NHS England, the Scottish Medicines Consortium (SMC), and other supporting partners such as Health Research Authority (HRA). The pathway places a target development profile approach (which is similar to the Trans-Pacific Partnership concept used by companies) at the core and this can help to identify pitfalls, break down silos, and support the allocation of expert resources where they add most value. Having NICE, SMC, and NHS England involved from an early stage is unique for ILAP and incorporates the needs of payers as well as regulators early in the process, thus improving the chances for a medicine to be taken up quickly by healthcare providers once approved, increasing patient access.

Many are called but few are chosen

During the first six months of operation, the MHRA received 32 applications with a representation of both large and small companies, including a university spin out and only a handful of innovation passports have been awarded. The applications covered common conditions such as lung cancer, community acquired pneumonia, chronic wounds, diabetes, but also rare diseases. The majority of projects are for products currently in late development stage, but over time, the MHRA is expecting the balance to shift to more early-stage development[2]. It is a unique feature of the ILAP, to have both the regulator and the HTA perspectives incorporated into the application for the Innovation Passport designation as well as criteria that ensure that the pathway is reserved for innovative medicines in high priority disease areas and indications, and the competition is fierce.

Mel Walker, Advisory Board Member at NDA, explains: "ILAP is unique in that it brings the HTA, the payer, and the regulator together at a much earlier stage in a products development when the entire clinical plan can be influenced in a way that is not possible when a product is further down the development pathway. By going early and involving all the right stakeholders, the chances of the product ultimately getting to patients, not just getting approved, can be maximised. It's a classic case of starting with the end in mind and companies should take a close look at this offering when developing early commercial strategy."

The ILAP Highway

The gateway for ILAP is the Innovation Passport which allows entry to the pathway very early, based on non-clinical data. To qualify for the pathway, an application must be made in which new medicines must demonstrate that they have the potential to offer significant benefits to patients with a life-threatening or seriously debilitating condition or meet a significant patient or public health need. It can be new vaccines, advanced therapy products, biologic, or chemical treatments or novel drug-device combinations, repurposed medicines or medicines for rare diseases[3].

If a developer fulfils the Innovation Passport criteria and is awarded an Innovation Passport designation, the next step is to submit a Target Development Profile (TDP). The TDP is a living document, similar to the WHO's Target Product Profile, defining key development and regulatory features, identifying potential pitfalls, and creation of a road map for delivering early patient access. It should also include details about how to work with other UK stakeholders for coordinated and efficient evidence generation and evaluation, whilst addressing commercial and access considerations. The TDP roadmap is important for the advancement of the product and should be updated along the development programme timelines as new data and evidence are generated. A team of experts will help define the TDP based on the product's characteristics.

While shaping the TDP, the developer will, together with the MHRA, NICE, and other partner colleagues, discuss the challenges and opportunities as well as what tools to use from a TDP Toolkit. The TDP Toolkit is a collection of activities and assessments to support the developer through the ILAP, such as adaptive inspections, actionable feedback on the common technical document (CTD), continuous benefit risk assessment integrating real world evidence, patient engagement, and assisted patient recruitment, along with Innovative and Flexible Licensing Routes. This means that the ILAP partners can provide support and guidance for the choice of licencing routes, for example rolling review, accelerated assessment. or a conditional marketing suthorisation as well as work-sharing pathways such as Access Consortium and Project Orbis. Among the initial Innovation Passport applications, eight oncology projects expressed interest in Project Orbis.

Patients have a say

The ILAP aims to involve patients in all aspects of decision-making and also offers an opportunity to embed the patient voice right from the beginning of the drug development process, through to regulatory decision-making and patient access. There are several stages within ILAP, where patient involvement is being developed enabling patients to influence the development and approval of products that will benefit them.

A pilot ILAP Patient Reference Group has been set up with members appointed by the MHRA, NICE, and SMC and provides valuable expertise and insights from the patient perspective throughout the process. Patient representatives are involved right from the start, even contributing to the evaluation of applications. This group will support the ILAP development to continuously improve how patients and patient representatives are involved in drug development, ensure that the patient experience is represented in the process, participate in the ILAP Steering group and also support the development of the ILAP Patient Engagement Tool[1].

According to the MHRA, there is ongoing work to develop a specific ILAP Partnership Agreement further mapping out the terms of delivering the different elements of the pathway (Innovation Passport, Target Development Profile, Tools of the Toolkit), and offer a blueprint for resource allocation and planning[1]. While the ILAP is already delivering a "single integrated platform" for collaborative working between the agency, partners, and the developer, the MHRA continues to raise the bar to optimise the process.

Where to start. As ILAP was just recently put into operation, merely knowing where to begin or which questions to ask can be the key to moving forward.

To help you prepare, ask yourself the following questions:

- Is the ILAP the right pathway for your product?
- Do you have the in-house resources and capacity to enable you to meet all the requirements of ILAP?
- Are you familiar with the options that are most advantageous for your product in the TDP Toolkit?

Our experts are on hand to work together with you and help create your regulatory strategy so that you can take full advantage of this unique, new pathway to market.

Contact us now: ***asktheexpert@ndareg.com***

References

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2. *Visit: assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/997365/MHRA_Board_Meeting_Pack_-_15_June_2021.pdf*
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