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UK Vaunts 'New Era' In Drug Approvals

Several New Routes To Market Are Now Available To Applicants

by Ian Schofield

The UK regulator, the MHRA, has published guidance on its new integrated pathway for innovative drug approvals, which it says is intended to bring together partners including health technology assessment agencies, health care bodies and patient organizations to offer “sustained collaboration” with drug developers.

The UK’s Medicines and Healthcare products Regulatory Agency has launched its new licensing and access pathway for innovative medicines that the agency says will prepare it for “a new era in medicines approvals in the UK.”

The Innovative Licensing and Access Pathway (ILAP), which was announced last year, is intended to reduce the time taken to get innovative drugs to market. It features a new medicines designation, the “innovation passport,” that will act as the gateway to the creation of a product-specific roadmap to guide drug development. (Also see "*MHRA Explains Entry Criteria for UK's New Innovative Medicines Pathway*" - Pink Sheet, 30 Oct, 2020.)

By involving NICE and the SMC – the health technology assessment bodies for England and Scotland respectively – as well as the National Health Service in England and NHS Improvement, the ILAP is expected to act as a single integrated platform for “sustained collaborative working” between the MHRA, its partners and drug developers.

Other organizations including the Health Research Authority and the National Institute for Health Research will also be involved in the pathway, as will patient organizations. “The patient voice will be integrated at every stage,” according to the MHRA, which is now a fully freestanding national agency following the end of the Brexit transition period and the provisional implementation of the new trade and cooperation agreement between the UK and the EU on 1

January.

“We are transforming the MHRA, making the regulator an enabler of innovation,” declared MHRA chief executive June Raine. The ILAP has “established new partnerships to robustly and safely support all new medicines at any point in their development, and most important of all, involve patients in all aspects of decision-making,” Raine said.

The new pathway will be supported by a "toolkit" of other measures, including tailored supervisory and licensing inspections, continuous benefit-risk assessment integrating real world evidence, innovative clinical trial design, a rapid clinical trial dossier pre-assessment service, rolling reviews, a national 150-day accelerated assessment procedure, and conditional and exceptional circumstances marketing authorizations.

> ***The ILAP offers “a genuine and significant opportunity to ensure new and innovative products reach patients across the UK, safely and quickly” – Health Improvement Scotland***

Steve Bates, chief executive of the UK BioIndustry Association, welcomed the implementation of the ILAP, saying the BIA supported a “joined-up life sciences ecosystem” that would allow the UK to become “a leading location for research and development of innovative medicines to the benefit of patients.”

His counterpart at the Association of the British Pharmaceutical Industry, Richard Torbett, said it was “really encouraging” to see the launch of the new pathway. Companies would be able to get new treatments to patients more quickly “by offering them a similar rolling review as was done with the *Pfizer*/*BioNTech*[COVID-19] vaccine and concurrent review by all parts of the health system,” Torbett noted.

For NICE chief executive Gillian Leng, partnering with the MHRA and others to build a “frictionless pathway to the timely availability of cost-effective medicines” was one of the ways NICE was “delivering benefits for patients, the NHS, and life sciences industry.”

A spokesperson for Healthcare Improvement Scotland, of which the SMC is a part, said the ILAP offered “a genuine and significant opportunity to ensure new and innovative products reach patients across the UK, safely and quickly.”

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ILAP & The Innovation Passport

Multiple entry points into the ILAP are available, depending on the stage of development of the product, the data available, the "ambition of the applicant to engage with UK stakeholders," and "the applicant's appetite for new innovative ways of working," the MHRA said.

It is open to drug developers at the preclinical study stage through to the mid-development program point, although applicants are "encouraged to apply early in the development of their products," the agency added. "Products that are towards the end of their development program are generally not suitable for the ILAP."

The innovation passport incorporates "broad and inclusive concepts of innovation and patient need" and encompasses a wide range of types of medicine, including advanced therapy medicinal products (ATMPs), orphan drugs and repurposed medicines.

> *"Applicants are strongly encouraged to include the views from patients or patient organizations around the benefits of a product in their evidence, if available" – MHRA*

The evidence required for a product to fulfil the criteria for the passport will depend on where the product is in the development pathway.

Generally speaking, the condition the product is intended to treat should be life-threatening or seriously debilitating, and there should be a significant patient or public health need. It should be an innovative medicine such as an ATMP, a new active substance or biological entity, or a novel drug-device combination, and be under development for a clinically significant new indication or for a rare disease or other special patient population.

The company should also be able to provide a summary of how patients are likely to benefit from the new product or indication compared with available therapeutic options. "Applicants are strongly encouraged to include the views from patients or patient organizations around the benefits of a product in their evidence, if available," according to the agency.

Securing the passport opens the way to the creation of a "target development profile" (TDP), a living document that will set out a roadmap for the development of, and patient access to, the new product. The TDP will make use of tools such as continuous benefit-risk assessment and more patient engagement in the development process, according to the MHRA.

All new drug applications, whatever the route chosen, are to be submitted through the MHRA submissions portal using the electronic common technical document (eCTD) format. "The MHRA will only recognize the MHRA Portal as an acceptable form of submission," the agency said. "All other forms of submission will be rejected."

Fees And Data Sharing

The fee charged for obtaining an innovation passport has initially been set at £3,624 ($4,960), while the creation of a TDP will cost companies £4,451. Both figures may be subject to change, the agency noted.

NICE's main contributions to the ILAP will be provided through its Office for Market Access and NICE Scientific Advice, and existing fee structures will be applied. "Fees from partner organizations may also apply," the MHRA added.

Any information shared during the ILAP will be considered confidential and held on a secure shared digital platform for access by the ILAP partners, "as agreed by the applicant," the MHRA noted. "In order to maximize the benefits of collaborative working with multiple UK stakeholders, the current and future sharing of relevant data is highly recommended."

A non-disclosure of information document/confidentiality agreement will be signed by the partners for each product that enters the pathway.

Other Guidance Documents

On 31 December, the MHRA published a set of more than 40 new and updated guidance documents, including details of the ILAP and the other new UK/Great Britain/Northern Ireland drug assessment pathways such as rolling review, accelerated assessment, and the "reliance" and "unfettered access" routes.

The documents also offer guidance in areas such as clinical trials, trade, the location of marketing authorization holders and qualified persons for pharmacovigilance, orphan medicines, converted EU centralized drug approvals, referrals, conditional and exceptional circumstances approvals, biosimilars and ATMPs, pharmacovigilance and pediatric medicines. These will be covered in upcoming articles in the *Pink Sheet*.