New Seat for EMA

One aspect of Brexit is currently Europe’s most discussed issue: the new seat of the European Medicines Agency. Landing the EMA is the main prize for the remaining EU-27 member states. At the closing date – 1 August 2017 - nineteen countries had officially lodged their application to host the EMA after the UK leaves the bloc in March 2019. Nominated cities: Amsterdam, Athens, Barcelona, Bonn, Bratislava, Brussels, Bucharest, Copenhagen, Dublin, Helsinki, Lille, Malta, Milan, Porto, Sofia, Stockholm, Vienna, Warsaw, and Zagreb.

These bidding documents, videos and websites include a variety of perks for the EMA, EMA staff members and their families, and the Union. Some cities offered to build a new building or provide rent-free use of a top location. Other cities cite the number of direct flights or their water quality. EU leaders will discuss the competition in October and there will be what has been billed in Brussels as a “Eurovision Song Contest-style Vote” by ministers in November. Each country can give three votes for its first preference, two for the second and one for its third. Any bid securing three points from 14 or more member states will be declared the victor. If this threshold is not met, there will be a second round for the top three cities. Each country gets only one vote. If there is still no winner, there will be a knock-out between the two final candidates.

EMA Preps for Brexit

The EMA has developed and initiated a business continuity plan to deal with the uncertainty and workload implications linked to Brexit. Noel Wathion, head of the Brexit task force, mentions challenges such as loss of experienced staff and running the business during relocation. To meet these challenges, a number of activities will be set on “hold,” including:

- Development of the European Medicines Web Portal, a new publicly-available online
Winds of Change Blow through Europe: Brexit, the EMA, Adaptive Pathways & PRIME

- Development of the European Medicines Web Portal, a new publicly-available online information source on all medicines marketed in the EU
- EMA’s contribution to the e-submission project that will allow applicants to electronically submit documents linked to authorisation requests for human and veterinary medicines in a secure and efficient way
- Development of a transparency roadmap for EMA that lays out future transparency measures for the Agency
- Participation in benchmarking medicines regulatory authorities in the EU as of 2018.

In addition, EMA reduced the number of audits as well as some corporate governance and support activities. Participation of EMA staff in external meetings or conferences has been reduced, as has the organisation of EMA meetings and workshops.

Adaptive Pathways

One of the EMA’s current projects is adaptive licensing, part of EMA efforts to improve timely access for patients to new medicines. Adaptive pathways is a scientific concept for medicine development and data generation which allows for early and progressive patient access to a medicine. The approach makes use of the existing EU regulatory framework for medicines.

Adaptive pathways are based on three principles:

- Iterative development, which either means:
  - Approval in stages, beginning with a restricted patient population then expanding to wider patient populations; or
  - Confirming the benefit-risk balance of a product, following a conditional approval based on early data (using surrogate endpoints) considered predictive of important clinical outcomes
- Gathering evidence through real-life use to supplement clinical trial data
- Early involvement of patients and health-technology-assessment bodies in discussions of a medicine’s development.

This concept applies primarily to treatments in areas of high medical need where it is difficult to collect data via traditional routes and where large clinical trials would unnecessarily expose patients who are unlikely to benefit from the medicine. EMA ran a pilot project between March 2014 and August 2016 to explore the practical implications of the adaptive pathways concept with medicines under development. The lessons learned were that not all medicinal products are good candidates for adaptive pathways and that generated data should be useful for both the competent authorities and the HTA agencies for reimbursing for these products on the basis of a conditional authorisation.

PRIME

Another important EMA project is PRIME (Priority Medicines). PRIME is a voluntary scheme launched to enhance support for the development of medicines that target an unmet medical need based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

Through PRIME, the EMA offers early and proactive support to medicine developers to optimise the generation of robust data on a medicine’s benefits and risks and enable accelerated assessment of
medicines applications. This will help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

Benefits for applicants to be included in PRIME are eligibility for accelerated assessment at the time of marketing authorisation application. Medicine developers can start regulatory dialogue in such an early phase that the design of the clinical trials can be improved to ensure that patients only participate in trials designed to provide the data necessary for an application.

Once a candidate medicine has been selected for PRIME, the Agency will:

- Appoint a rapporteur from the Committee for Medicinal Products for Human Use (CHMP, or from the Committee on Advanced Therapies [CAT] in the case of an advanced therapy) to provide continuous support and help build knowledge ahead of a marketing authorisation application
- Organise a kick-off meeting with the CHMP/CAT rapporteur and a multidisciplinary group of experts, so that they provide guidance on the overall development plan and regulatory strategy
- Assign a dedicated contact point
- Provide scientific advice at key development milestones, involving additional stakeholders such as health technology-assessment bodies, to facilitate quicker access for patients to the new medicine
- Confirm potential for accelerated assessment at the time of marketing authorisation application.

This scheme focuses on medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients who have no treatment options. These medicines are considered priority medicines by EMA. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.