The STAMP of approval for early access to new drugs

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It is clear that developing a new medicine is a lengthy process. In November 2014, the Tufts Center for the Study of Drug Development estimated the average time taken from synthesis of a new compound to regulatory approval of an application for marketing authorisation to be 128 months – almost 13 years. (See 'Cost of Developing a New Drug', 18 November 2014.) When this is coupled with the increased access that patients have to information on new drug development, for example through patient organisations, social media, pharmaceutical company websites and regulatory documentation, it is not surprising that there is pressure on industry and regulators to allow patients access to medicines in a timely, and sometimes early, manner. This pressure is welcomed by industry, which is keen to allow patients access to new medicines, and it has been taken on board by regulators who have devised a variety of mechanisms to allow this access to take place. Our focus this month is on this very important area of early/timely access to new medicines.

To give us some insight from a patient perspective we have an article from three members of the European Medicines Agency's Committee for Orphan Medicinal Products (COMP), each of whom represents different rare disease patient organisations (the European Genetic Alliance Network, European Organisation for Rare Diseases and Cystic Fibrosis Europe, respectively). Their article discusses the importance of early access to medicines for patients suffering from rare diseases, and highlights the various aspects which are of particular importance for rare diseases as opposed to more common diseases.

Regulators around the world have taken on board this need for timely/early access to new medicines, most recently in the EU with the Safe and Timely Access to Medicines for Patient (STAMP) expert group established by the European Commission, and the EMA's proposed Priority Medicines (PRIME) scheme. In this issue, we have two further focus articles looking at schemes which are available in Europe (focusing on the UK) and North America (US and Canada). An article from a regulator's perspective is provided by the Medicines and Healthcare products Regulatory Agency (MHRA). This discusses the UK's Early Access to Medicines Scheme (EAMS), which gives patients with life-threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need.

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Our North American article looks at the market access and expedited regulatory review programmes available in this region. Market access programmes include expanded access (US) and the special access program (Canada). Expedited regulatory review programmes discussed include the accelerated approval pathway, the fast track and breakthrough therapy designations (US) and priority review (US and Canada).

In addition to our focus articles, we also have articles which will be interesting for developers of different types of product at different stages of development. First, we have an article from the EMA's SME Office which provides some fascinating EMA insights on marketing authorisations, regulatory assistance and briefing meetings for SMEs in the biologics and advanced therapies fields. This is followed by an interview with Anja Holm of the Danish Medicines Agency, who is also Chair of the Committee for Veterinary Medicinal Products (CVMP) and has been involved in many areas of the regulation of medicinal products. Finally, we have a meeting report which provides an update following two recent webinars hosted by the EMA on the new functionality of the PSUR repository.