Medicine's Adaptive Pathways for Patients: MAPPs

What are Medicines Adaptive Pathways to Patients (MAPPs)?

MAPPs refer to flexible development and access pathways within the current regulatory framework that balance early patient access, public health and societal benefits. MAPPs start with an early authorisation of a product focused on a well-defined and targeted population for whom benefits are likely to outweigh risks. The target population is adjusted as the evidence base expands. MAPPs may integrate adaptive clinical trial design, patient centric benefit/risk assessments and continuous re-evaluation as new evidence becomes available. MAPPs, therefore, relate to the entire life cycle of a medicine from development, through licensing to patient access (reimbursement and healthcare delivery).

Why are MAPPs important and for whom?

Patients are demanding faster access to new medicines and the regulatory environment is lagging behind evolving science. Conventional R&D models are no longer financially viable and have become a major hurdle to efficient drug development, while general response rates to modern medicines are not satisfactory. A more flexible pathway would not only accelerate patient access to crucial therapies but would also increase their probability of success as the therapies would be given to those patients most likely to respond. The cost for both industry and healthcare providers could also significantly decrease.

However, a pre-requisite for the success of the implementation of MAPPs lies in the full and common understanding of its value, not just for industry, but across the entire innovation life cycle: for regulators, HTAs, payers, governments, clinicians and, most importantly, patients.

How will MAPPs work?

- MAPPs will initially focus on targeted, stratified medicines with clear biomarkers, well-defined populations, preferably an available diagnostic, and high level of efficacy and safety
- MAPPs imply an initial marketing authorisation for a limited patient population upon demonstration of a positive benefit/risk balance
- It will generate confirmatory evidence from observational data sources while randomised clinical trials continue in support to the original or additional indications
- As more indications are validated, the population approved for the new medication will adapt to the evolving evidence base, as will the initial pricing and value assessment.

The evidence generation plan would be established in advance through joint Scientific Advice involving at least regulators and HTA/payers, or using other mechanisms for collaborative development or market access conversations. This process would include:

- Setting the data requirements for early regulatory approval based on initial evidence
- Iterative benefit-risk evaluations at pre-agreed milestones to:
    - Confirm initial findings
    - Consider expansion of indicated population using evidence from real world and parallel clinical development
    - Consider re-assessment of value as longer-term effectiveness and outcomes are understood
- Agreement on evidence package and methodologies for re-assessment

What are the challenges?

As MAPPs intends to bring therapies to patients earlier, it must rely on an infrastructure of validated stratification tools capable of collecting data, identifying target patient groups and tracking the impact of specific medicines on their health. Current challenges include:

- The early, collaborative agreement by stakeholders (patients, regulators, HTA/payers, practitioners, industry) on the evidence package required for early regulatory approval, reimbursement, and access for patients
- The willingness of patients, payers and industry to operate with increased uncertainty
- The IT infrastructure needed to provide the real world evidence base

How will MAPPs be implemented?

Although already discussed in many public fora and supported by patients, regulators and the academic community, MAPPs is not yet clearly defined; its feasibility within the current legislative framework and its viability for all stakeholders must be demonstrated and quantified. Three instruments are available for this purpose:

- IMI multi-stakeholder coordination and stakeholder dialogue initiatives
- IMI research projects to develop missing enablers of MAPPs (tools and methodologies including for modelling impact on all parties)
- EMA Adaptive Licensing Pilot project ('Safe Harbour') – a regulator led forum to simulate adaptive regulatory pathways for example live assets.

Further reading:
http://www.nature.com/clpt/journal/v91/n3/full/clpt2011345a.html
http://www.nature.com/nrd/journal/v12/n4/full/nrd3981.html