European Federation of Pharmaceutical Industries and Associations

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EFPIA's position on expedited regulatory pathways

Innovative and novel therapeutic treatment options are in great demand around the globe and are essential in bringing significant health benefits to society and patients. Regulators face significant challenges as treatments become more innovative and scientific development becomes more tailored especially in areas of high unmet medical need. Aligned and science-driven regulatory standards provide assurance of quality, safety, and efficacy and are important in making treatments available in a timely fashion. Equally important are efficient regulatory pathways that enable good decision making and best use of limited agency and industry resources.

There are good examples of agencies around the globe speeding up access to medicines which fulfils the national requirements for unmet medical need e.g. Expedited Regulatory Pathways such as US FDA's Breakthrough Therapy and Accelerated Approval, PMDA's Sakigake and EMA's PRIME and Accelerated Review. This paper focuses on how other agencies may approach such products for which approval is sought based on a positive benefit/risk assessment with limited data package and thus more uncertainty than standard approval pathways necessitating post-approval follow-up studies. The strategy chosen by each regulatory agency depends on its overall approach, capabilities and resources as well as the local legal framework.

EFPIA supports the paper on regulatory reliance[1] published by IFPMA[2] and emphasises that the reliance principles outlined in the IFPMA paper also apply for products which have undergone expedited development and /or expedited regulatory pathways and might have an abbreviated data package at the time of filing.

Examples of expedited regulatory pathways

Some Regulatory Authorities have already established regulatory pathways to achieve fast approvals through one or more of the following strategies:

- Expedited development: This involves close interaction and dialogue between sponsor and the agency to guide evidence generation strategies that lead to earlier regulatory approval. This may

[1] WHO defines **Reliance** as the act whereby the NRA in one jurisdiction may take into account and give significant weight to – i.e., totally or partially rely upon – evaluations performed by another NRA or trusted institution in reaching its own decision. The relying authority remains responsible and accountable for decisions taken, even when it relies on the decisions and information of others.
https://www.who.int/medicines/areas/quality_safety/quality_assurance/GoodRegulatory_PracticesPublicConsult.pdf

[2] https://www.ifpma.org/resource-centre/ifpma-position-paper-on-regulatory-reliance


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include abbreviated data package or faster accrual of required data to speed up the data generation cycle during product development.

- Expedited submission: Allow dossier components and documents to be submitted as soon as they are prepared to enable regulators to start registration procedures / review of documents and data before official start of the regulatory approval procedure (e.g. rolling submission)
- Expedited review: Shorter regulatory review time during the approval procedure

EFPIA recommendations for expedited regulatory pathways

When a regulatory agency is considering a product that addresses an unmet medical need and for which approval may be based on limited data package including advanced therapy products and/or use of novel evidence generation principles, the following should always be considered in order to speed up the availability of the new therapy to local patients:

- Consider specific designation from reference agency (Breakthrough Therapy, PRIME, Sakigake etc.) to determine if product meets a local unmet medical need
- Consider waiving certain country specific requirements like provision of registration samples, local pre-approval testing, GMP inspections, generation of local clinical data etc.
- Rely on reference agency post-approval commitments unless specific local commitments are scientifically justified
- Ensure local robust Pharmacovigilance monitoring
- Have a system in place which enables completion of post-approval commitment submissions and appropriate regulatory actions. Sponsor/developer must commit to share all relevant post-approval data sent to the reference agency

If a reference agency has already approved the product or is in the process of doing so, the simplest way to expedite the approval may be to:

- Rely on the reference agency assessment and perform an abbreviated review focussing on the applicability of the results to the local population and health care context

For agencies seeking to develop a stand-alone expedited regulatory pathway:

- Seek to harmonise national requirements with existing expedited pathways available globally e.g. Breakthrough Therapy Designation, PRIME, Sakigake
- Ensure engagement opportunity with local agency during development and review to discuss specific requirements
- Ensure appropriate resources, capacity and expertise for communication during development and review


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This figure shows the considerations which an NRA is recommended to follow when contacted by a sponsor proposing expedited pathway for a product: