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Transforming Regulatory Pathways: A Path to Swift Patient Access (EFPIA Guest Blog)

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In today's fast-paced world of medical advancement, regulatory frameworks have a crucial role to act as a key enabler for timely access to innovation for patients, ensuring sustainable supply of medicines, boosting Research & Development investment and enabling economic growth.

With the final discussions of the EU Pharmaceutical Legislation taking place, prioritising changes to the regulatory framework so that the EU maintains its leadership in regulatory excellence is vital, not only for patients, but for EU competitiveness. Current concerns are that some of the proposed legislation lacks a vision of how the EU could benefit from an improved regulatory framework in a global context and may result in a missed opportunity. Further improvements, especially related to the expedited regulatory pathways, as well as the need for simplification, should be considered.

Why it matters

The regulatory framework determines the timely start up of clinical trials in the EU, the licensing of new products and the requirements for the manufacture and supply of the products. The current framework has additional hurdles compared to other global markets such as; complex licensing procedures for clinical trials; restricting expedited regulatory pathways to highly selected products and additional requirements for manufacturing of pharmaceuticals in the EU. If we miss this opportunity to introduce agility and make the regulations future-proof this could ultimately lead to the EU falling behind other global players with a significant impact on European patients having timely access to innovative medicines. Other unintended consequences are public clinical research institutions falling behind in clinical research compared to their global peers.

Learning from others

Regulatory reforms introduced in China's 12th Five-Year Plan played a crucial role in supporting the country's biotechnology industry as part of its goal for sustainable economic growth[i]. Since 2015, the National Medical Products Administration has been aligning medicine approval processes with global standards[ii], significantly reducing approval timelines for example for clinical trials for new medicines from just over two years to [iii] 60 days[iv]. Efficiencies encouraged a shift from generics manufacturing to a highly innovative pharma industry[ii], fostering an environment ripe for innovation and attracting global investments. China now ranks second globally in terms of innovative drug contributions to the global pipeline[v]. By creating a robust end-to-end regulatory framework for pharmaceutical research and industry, these changes not only enhanced China's position in the global biotech market but also improved patient access to innovative therapies[v], reinforcing the connection between effective regulation, economic advancement and patient benefit.

Japan assessed its regulatory framework following a significant decline in approvals of medicines. In 2023, 143 new medicines were not available to Japanese patients with no plans for 60% of them to be developed for the market[vi]. The phenomenon of "drug loss," where approved drugs in major markets are not developed in Japan[vii],

underscores the negative impact regulations can have on patients access to innovative treatments especially for smaller biotech companies. Japan's Pharmaceuticals and Medical Devices Agency now promotes its reformed regulatory systems, including an enhanced innovative drug designation program that provides priority reviews and a conditional approval system that allows for early access in instances where conducting confirmatory clinical studies is challenging[vii].

Timely access to innovation for EU patients

To avoid so-called drug lag (delayed approval in EU)/drug loss (not developed in the EU), the following levers should be considered: reducing timelines, opening up expedited pathways and reducing overall complexity of the framework for clinical trials, licensing of new medicines and manufacturing, which we have seen from both the US and China can attract investments in R&D and manufacturing, ultimately leading to patient access to innovation.

Reducing approval timelines

Efficient regulatory timelines were part of the European Commission's original proposal. If Standard Assessment timelines are reduced to 180 days and the time for the Commission to issue the Marketing Authorisation are reduced to 46 days, (as per the original Commission proposal[viii]) or even reduce this administrative step to 30 days, this would see a reduction in patient waiting times of one and a half months. While this may not seem a tremendous gain in timelines, a reduction of one and a half months is not to be underestimated as it means patients can start new or improved treatments faster which may enable them to take on social/work activities again or even make the difference between life and death.

Greater use of Expedited Regulatory Pathways.

The EU's Expedited Regulatory Pathways are designed to speed up the approval processes for new medicines where there is an unmet medical need, ultimately enabling patients quicker access to essential treatments e.g. for orphan diseases impacting very few patients or cancers where no treatment is currently available. Given the complexity of innovative medicine development, broader eligibility of treatments to these pathways is essential, allowing a wider interpretation of the concept of unmet medical need would be one way to allow more treatments to use these pathways. It is essential to create a more patient-centric definition of unmet medical need which emphasises not only the presence of a medical condition, but also improvements to a person's quality of life. Medicines should enhance daily living for patients, especially those with severe chronic diseases.

Expedited pathways are used far less in the EU. From 2019 to 2023, only 8% of new active substances in the EU utilized these pathways whereas in the US it was 71%[ix]. This indicates a pressing need for the EU to bolster its Expediated Regulatory Pathway framework to remain competitive.

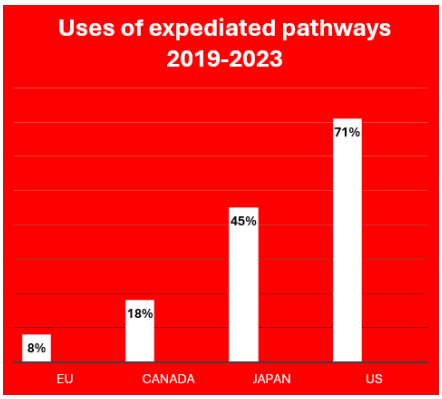

Figure 1

The US expedited regulatory pathway tools are better interconnected with each other; i.e. once you get Breakthrough Designation (expedited development and review of drugs for serious or life-threatening conditions)

you often get “Priority Review” (an accelerated assessment, experience shows it to be 4 months shorter), Rolling Review and Accelerated Assessment (equivalent to Conditional Marketing Authorisation in EU). In the EU you have to apply to get Priority Medicines Scheme designation then re-apply separately to get Accelerated Assessment and re-apply again to get Conditional Marketing Authorisation or Phased Review. It’s time to build simpler pathways for new medicines.

> *“We need to reduce the complexity of the framework for research, manufacturing and supply”.*

Streamlining Paediatric Launch Obligations

The ambition to have paediatric versions of products available (e.g. oral solutions in addition to tablets) is shared with the Commission, the current proposals to ‘place on the market’ in the conventional way in all Member States individually [viii], could mean companies face severe practical, stock and supply issues when there may be very limited demand for the treatment. Amending the obligation to place the medicinal product on the ‘Union market’; making the product available for paediatric patients in all Member States (where the adult medicinal product is already placed on the market) in a pragmatic manner using flexible pathways that already exist today under the current law would avoid disproportionate and wasteful stocking obligations in countries where there might be very limited or even no demand for the paediatric product. Additional flexibility in terms of local language labelling would also facilitate addressing supply chain challenges for low volume products and provides a good balance between logistical feasibility and available of the paediatric medicines where there is a real need.

The moment for action is now

The trilogue discussions on revision of the Pharmaceutical Legislation represent a golden opportunity for the EU to restore the competitiveness of the EU’s pharmaceutical landscape: by enhancing expedited pathways, refining paediatric research requirements and streamlining timelines, the EU can build a regulatory environment that not only fosters innovation but also prioritizes patient access. The call to action is clear: legislative improvements must be embraced to revitalise Europe’s place in the global health landscape

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[ix] New drug approvals in six major authroities 2014-2023 Available at https://cirsci.org/wp-content/uploads/dlm_uploads/2024/07/CIRS-RD-Briefing-93-six-agency-briefing-v2.0.pdf Last Accessed June 2025

Figure 1. New drug approvals in six major authroities 2014-2023 Available at https://cirsci.org/wp-content/uploads/dlm_uploads/2024/07/CIRS-RD-Briefing-93-six-agency-briefing-v2.0.pdf Last Accessed June 2025

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