PINK SHEET
CITELINE REGULATORY

16 Oct 2015 | **News**

Faster EU Drug Assessment Pathway Could Launch In First Quarter 2016

by Neena Brizmohun

Hoping for 'convergence' with FDA's breakthrough program, EU's PRIME designation could be offered to subset of medicines that qualify for current accelerated assessment pathway.

The European Medicines Agency's program to speed development of early-stage innovative medicines faces two big inflection points in the coming months: meeting with FDA officials to understand what has made the "breakthrough" designation such a hit, and seeking formal feedback from industry on the EMA's own plans.

The EMA program, called PRIME (for priority medicines), is being designed to provide companies developing such drugs with enhanced regulatory and scientific support so as to optimize product development and enable faster regulatory assessments (*EMA Seeks Industry Feedback On New Scheme For Supporting Drugs For Unmet Needs* – *Scrip Regulatory Affairs*, Sept. 30, 2015).

A draft version of the scheme is being drawn up and could be ready for a two-month public consultation by the end of October, the European Commission's Sabine Jülicher told delegates at the recent TOPRA annual symposium in Berlin.

PRIME is expected to be similar to the US breakthrough therapy designation program, which aims to expedite the approval of products that could be significant advances over existing treatments. The FDA designation, which has proven more popular than expected since it was created in 2012, offers sponsors frequent consultations with senior agency staff (Also see "*Breakthrough Designation Is A Two-Way Street, FDA Cautions Sponsors*" - Pink Sheet, 29 Jun, 2015.).

To ensure "convergence" with the US program, EMA officials are planning to meet up with breakthrough therapy experts from FDA shortly to compare both schemes. Alignment of the programs would not only facilitate more timely access to medicines, it would also help support

global drug development.

Following the close of PRIME's public consultation, Jülicher expects that the new scheme could be ready for launch in the first quarter of 2016.

Smarter Use Of Accelerated Assessment

PRIME is not an entirely new concept. It will build on, but not replace, the EU's existing accelerated assessment authorization pathway for drugs for unmet needs, which has been in place for many years but has come under criticism for failing to meet its intended use (*EMA Seeks To Optimize Use Of Accelerated Assessment And Conditional Authorization Pathways* – *Scrip Regulatory Affairs*, July 28, 2015).

Under the accelerated assessment, the scientific assessment time may be reduced from 210 days to 150 days.

PRIME is expected to apply to a subsection of medicines that qualify for the current accelerated assessment pathway, introducing mechanisms that will make it easier for sponsors to develop those medicines.

One such mechanism could involve assigning a regulatory agency expert, or rapporteur, to a product throughout its development. As with US breakthrough designations, under which the FDA is supposed to ensure sponsors receive timely advice and interactive communications to help them design and conduct drug development programs as efficiently as possible, the EU rapporteur would give PRIME designees advice throughout their programs.

For Medicines Of 'Major Interest'

The new scheme is also expected to include discussion relating to health technology assessment (HTA). "We need to see beyond authorization only," Jülicher said, explaining that there was little point in speeding up market authorization only to then lose time gained later on because of concerns relating to HTA.

PRIME basically provides "a smarter way" of using tools that are already available in the EU.

As for the criteria for designation under PRIME, this is still under discussion, said Jülicher, who is head of unit D5 (Medicinal Products – Authorisations, EMA) at the commission's Directorate General for Health and Food Safety.

It will be based on the criteria of the current accelerated assessment pathway – which is used for medicines that are of "major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation." But it will not be 100% the same, Jülicher told *Scrip Regulatory Affairs*.

The commission's new expert group, STAMP (Safe and Timely Access to Medicines for Patients), is expected to discuss the criteria and clarify what would justify a major public health interest at its next meeting, which is scheduled for Oct. 20.

The Need For The New Scheme

As for the drug industry's reaction to PRIME, EU trade group EFPIA said it was "looking forward" to the new scheme.

Speaking at the TOPRA symposium, EFPIA's director of regulatory affairs, Sabine Atzo, expressed hope that the scheme would serve to foster development and innovation for priority medicines.

Atzo also said that EFPIA wanted to see a "broad alignment" of the criteria for PRIME with the US breakthrough designation "to support global development" and the possibility of a parallel process. In addition, it should be possible to adapt PRIME quickly as experience with the scheme evolves.

PRIME, which EMA proposed earlier this year (under the name of pathfinder), follows the EMA's much hyped adaptive pathways initiative, which is also using existing regulatory mechanisms in a paradigmatic way to accelerate drug development and review to deliver innovative medicines for unmet needs to patients faster.

The concept of adaptive pathways foresees either an initial approval in a well-defined patient subgroup with a high medical need and subsequent widening of the indication to a larger patient population, or an early regulatory approval (e.g., conditional approval) which is prospectively planned, and where uncertainty is reduced through the collection of post-approval data on the medicine's use in patients.

The adaptive pathways initiative is currently being tested in a pilot project (*A Lot Of Promise But A Long Way To Go: An Initial Assessment Of The Eu Adaptive Pathways Pilot* – *Scrip Regulatory Affairs,* July 1, 2015).

Explaining the need for these new schemes, Jülicher explained that "there is still a perception that the development of innovative medicines is complex and costly," despite the availability of the existing accelerated assessment pathway and the other EU programs for expediting access to drugs, including conditional marketing authorization, authorization under exceptional circumstances and the compassionate use scheme.

As for the accelerated assessment and conditional marketing authorization pathways, EMA in July proposed improvements relating to their use.

Specifically, the agency issued for public consultation revised guidelines for both fast-track

routes in a bid to optimize their use by drug developers and consequently allow more medicines for treating unmet medical needs to reach patients earlier.

Regarding accelerated assessment, drug companies have expressed multiple complaints about the pathway and the way in which EMA has used, or has appeared to use, it. For example, there appeared to be a high hurdle for EMA acceptance of requests to use the pathway; that EMA itself discouraged its use; and that even when accelerated assessment was requested, it was often declined and/or revoked during the assessment phase.

[Editor's note: A version of this article is also published in Scrip Regulatory Affairs. "The Pink Sheet" DAILY brings selected complementary coverage from sister publications to subscribers.]