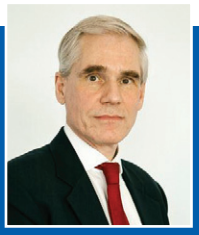
A Life-Span Approach to Decision Making: Why EU Collaboration Is a *Sine Qua Non*

Hans-Georg Eichler, MD, MSc, Senior Medical Officer, European Medicines Agency, London, UK



KEY POINTS . . .

A "life-span" approach is needed to enable translation of beneficial innovation to current and future patients.

Collaboration across and within decision-maker groups is a *sine qua non.*

Health technology assessment (HTA) Joint Action 3 (JA3) will hopefully be the catalyst for much-needed collaboration.

This is one of three articles in this issue on the topic of decision making on health care technologies. Dr. Eichler's article examines an adaptive pathways approach via EU Collaboration.

I would like you to meet two people. Jane is a woman in her late 50s and was recently diagnosed with advanced cancer. John, who is also in his late 50s, is in good health, but knows that he has a high chance of receiving an eventual cancer diagnosis—and may likely die of cancer, given his family's history with the disease.

Jane, who has exhausted all of her treatment possibilities and has relapsed, fears that her time may be running out. John believes that he has at least another 15-20 years remaining. Both Jane and John learn of a promising new cancer treatment, but as with many experimental therapies, the treatment comes with some associated risks and uncertainties. Given the urgency of her situation, Jane is keen to get access to the new treatment and is willing to accept the risks and uncertainty of the new treatment. John, on the other hand, believes that the treatment should be well-tested before it is made available to him; so he is unwilling to accept the uncertainty of this new treatment.

The 'Life-Span' or 'Adaptive Pathways' Approach

Should our health care systems be catering to Jane's needs, or to John's? Or to the needs of both?

If you feel that the health care systems should cater to both Jane and John, you have just endorsed a 'life-span' approach for bringing drugs to market. A life-span approach, also known as 'adaptive pathways' is the only realistic way to balance the needs of current patients with the needs of future patients.

Adaptive pathways aim to improve timely access for patients to promising new medicines; the concept is based on:

1. Approval in stages—beginning with an initial, restricted patient population then expanding to wider patient populations.
2. Progressive reduction of uncertainty—refining knowledge about the benefits and harms of a product in different subgroups of patients.
3. Gathering on-market evidence throughout the product life-span by way of collecting real-world data to supplement clinical trial data.
4. Early involvement of patients and health technology assessment bodies in discussions of a drug's development to inform value judgements about timely licensing and reimbursement.

A life-span approach, also known as 'adaptive pathways' is the only realistic way to balance the needs of current patients with the needs of future patients.

From a regulators' perspective, the adaptive pathways approach builds on regulatory processes already in place within the existing EU legal framework. These include scientific advice; compassionate use; the conditional approval mechanism; as well as patient registries and other pharmacovigilance tools that allow collection of real-life data and facilitate the development of risk-management plans for each medicine.

Collaboration Is Needed

Unlike Jane who has already undergone treatment for a serious illness, many of us will eventually have to deal with a life-threatening illness. And when that moment arrives, we all—like Jane—would prefer that promising drug candidates make their journey to the marketplace as quickly as possible. However, all decision makers (i.e., regulatory agencies, HTA bodies, payers, prescribers, and patients [Figure 1]) involved in the journey have to be in alignment to achieve that goal; otherwise, the journey cannot be completed. Collaboration across and within these

decision-maker groups in EU member states is a *sine qua non*.

Limited Resources and Other Concerns

Why is collaboration on adaptive pathways sometimes difficult to achieve? First, all organizations have limited resources, and engaging in repeat multi-stakeholder dialogues with drug developers is resource-intensive.

Second, collaboration requires at least some degree of alignment of evidence standards.

Third, some organizations are not sure that they even want to take a life-span approach. That said, others are willing to explore this course, and we've seen several successful and constructive discussions surrounding the multi-stakeholder adaptive pathways pilot projects held at the European Medicines Agency. It gives reason for cautious optimism that at least some EU payer organizations are now willing to familiarize themselves with and explore the adaptive pathways concept.

Turning the 'Half-Moon' to a 'Full Moon'

To broaden participation and acceptance of the adaptive pathways concept, we hope to have more EU member states engaged. So far, the picture of participation resembles somewhat of a "half-moon," with participation coming mostly from northwest Europe. However, we hope to get the "full moon" of Europe engaged. Specifically, we have to bring in payer organizations.

We see the Health Technology Assessment Joint Action 3 (HTA JA3) as a potential catalyst and a great opportunity for further collaboration in Europe. The adaptive pathways concept is on the agenda of the JA3. Additionally, the collaborative infrastructure will encompass data sharing and standardization, as well as work with registries. Adding these elements to the life-span approach—or adaptive pathway—is needed to enable the translation of beneficial innovation to both current and future patients. ■

Additional information:
This article is based on a presentation at the plenary session, "Strategy in Motion: The Current and Future Lifecycle Approach to Decision Making on Health Technologies," given at the ISPOR 18th Annual European Congress, Milan, Italy 9 November 2015. The following two articles from Finn Børlum Kristensen (page 12) and Mirella Marlow (page 14) were also taken from this session.

To view Dr. Eichler's presentation, go to: http://www.ispor.org/Event/ReleasedPresentations/2015Milan

Figure 1. Why is collaboration in the EU a *sine qua non?*