

Meeting summary

EUROPEAN HEALTHCARE INNOVATION LEADERSHIP NETWORK
PILOTS OF MULTI-STAKEHOLDER CONSULTATIONS

2 SEPTEMBER 2010

Piloting a new multi-stakeholder approach to consultations in early-stage drug development

Introduction

On 14–15 July 2010, leaders of healthcare institutions from five Member States and the European Union (EU) convened in London to launch a new pilot process testing multi-stakeholder consultations in early-stage drug development. The pilots will involve clinicians, health technology assessors (HTAs), patient representatives, payers, regulators and drug developers across multiple EU Member States. Participating companies will seek early advice regarding a pipeline medicine in either breast cancer or type 2 diabetes. The purpose of the consultations is to improve clarity and alignment among diverse stakeholders regarding what constitutes a medicine's value and the evidence required to demonstrate that value most effectively. Over 30 participants from agencies representing five countries and pan-European organisations were present at the launch meeting, as well as representatives of the Breast Cancer and Type 2 Diabetes Working Groups (discussed below). [Appendix 1 contains a list of institutions contributing to the pilots.](#)

The pilot launch meeting was a milestone along a multi-year process involving over 100 healthcare leaders across Europe. The key driver of this process has been the European Healthcare Innovation Leadership Network (the Network). The Network is comprised of a group of healthcare leaders committed to improving health outcomes by ensuring continued access to value-adding treatments addressing unmet medical needs. The Network has sponsored two disease-specific Working Groups to develop Shared Value Frameworks for breast cancer and type 2 diabetes. The recommendation for pilots of multi-stakeholder consultations in early-stage drug development grew out of the Working Groups' activities. [An overview of the Network and the road to the pilots is provided in Appendix 2.](#)

At the launch meeting, pilot participants affirmed the need for enhanced collaboration among stakeholders prior to the regulatory and reimbursement review process in order to support a more sustainable development path for innovative medicines. They noted the uniqueness of the occasion, as it was *“the first time that so many people coming from different horizons were around the same table.”* Participants noted the opportunity to *“move towards defining a common set of objectives from a development programme that will meet everyone's needs and hopefully result in simplifying the development process to make good products available to patients more quickly.”*

Launch meeting participants agreed a high-level process design for the pilots. They discussed expectations and measures for success, and raised a number of issues that remain to be addressed in order to support the successful completion of the pilots. The meeting concluded with agreement to conduct pilot consultations later in the year with committed medicines from participating companies. Plans are to conduct the first consultation in September–October of this year, the second in October–November and the third in November–January.

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The launch meeting comprised a mixture of plenary discussion and focused work in breakout groups, preceded by one-to-one conversations prior to the meeting between Tapestry Networks and participants to obtain individual perspectives. A modified version of the Chatham House Rule was used throughout the day, whereby names of participants and their affiliations are a matter of public record, but comments made during meetings are not attributed to individuals or organisations. This document summarises the day's discussion and shares the path forward for testing multi-stakeholder consultations in early-stage drug development. [Table 1 lists the attendees of the launch meeting.](#)

Table 1: Launch meeting attendees

European Medicines Agency (EMA)

- **Eric Abadie** | EMA
- **Hans-Georg Eichler** | EMA | T2DWG
- **Bruno Flamion** | EMA
- **Patrick Le Courtois** | EMA
- **Spiros Vamvakas** | EMA

Regulators

- **Kristina Dunder** | Medical Products Agency (Sweden)
- **Harald Enzmann** | EMA | BCWG
- **Ian Hudson** | Medicines and Healthcare Regulatory Agency (United Kingdom)
- **Bertil Jonsson** | MPA (Sweden) | BCWG

National HTAs/payers

- **Pietro Folino Gallo** | Italian Medicines Agency (AIFA), (Italy)
- **James Gossov** | NHS | Stockton-on-Tees Primary Care Trust (United Kingdom)
- **Niklas Hedberg** | Dental and Pharmaceutical Benefits Agency (TLV) (Sweden)
- **Carole Longson** | NICE (United Kingdom)
- **François Meyer** | Transparency Commission (France)
- **Ad Schuurman** | Dutch Health Care Insurance Board (CVZ) (The Netherlands)

Breast Cancer Working Group (BCWG) representatives

- **Jonas Bergh** | Karolinska Institute (Sweden) and Manchester Hospital (United Kingdom)
- **Anthony Howell** | Christie NHS Foundation Trust (United Kingdom)

Type 2 Diabetes Working Group (T2DWG) representatives

- **Jean-François Bergmann** | University Paris VII (France)
- **Philip Home** | University of Newcastle upon Tyne (United Kingdom)
- **Peter Kolominsky-Rabas** | German Centre for Public Health (Germany)
- **Eberhard Standl** | Munich Diabetes Research Institute (Germany)

Patient advocates

- **Pauline Evers** | NFK, Dutch Federation of Cancer Patient Organisations and BVN, Netherlands Breast Cancer Association (The Netherlands)
- **Susan Knox** | EUROPA DONNA | Breast Cancer Working Group | *Observer*
- **Arja Leppänen** | BRO, Swedish Breast Cancer Organisation (Sweden)
- **Maarten Ploeg** | Dutch Diabetes Association | T2DWG (The Netherlands)

Industry

- **Mats Mårfält** | AstraZeneca
- **Gunnar Olsson** | AstraZeneca
- **Mark McClung** | GlaxoSmithKline
- **Carlo Russo** | GlaxoSmithKline
- **Reshma Patel** | Johnson & Johnson
- **Margaret Yu** | Johnson & Johnson

Executive summary

The launch meeting resulted in the following outcomes:

Participants affirmed the need for pilots of multi-stakeholder consultations in early-stage drug development

Participants identified a number of challenges in the drug development process that limit health systems' ability to deliver the right medicines to the right patients at the right time. These difficulties include the high cost of bringing innovative new medicines to market; the limited additional benefit over existing treatments that many new medicines provide; and the delay in getting newly-developed medicines that do promise significant improvement in health outcomes to patients in a timely manner. Participants believe that multi-stakeholder consultations in early-stage drug development can help address these problems by improving clarity and alignment among diverse stakeholders regarding what constitutes a medicine's value, along with what evidence is required to most effectively demonstrate that value. This clarity and alignment, in turn, can focus development programmes on providing the medicines that health systems need most. Of similar importance, greater transparency regarding stakeholder expectations promises to reduce the number of late-stage failures in drug company pipelines, thereby removing a significant source of cost from the drug development system.

Participants agreed pilot design principles

Tapestry Networks proposed a set of pilot design principles drawn from experience with existing early consultation processes. Launch meeting participants considered and discussed these principles and agreed that the pilots should create an opportunity for mutual learning among participating institutions through greater transparency and collaboration. They agreed that this outcome was most likely if the pilots were constructed to provide medicine sponsors with non-binding advice regarding the methodology of value assessment through an open dialogue in which all participants enjoy equal standing.

Participants expect that the opportunity to engage in pilot consultations, if successful, will be made available to other pharmaceutical companies as well. Participants deferred the question of how such consultations should be institutionalised until after the pilots are completed and evaluated.

Participants agreed a high-level process for the pilots

Consistent with the design principles noted above, the launch meeting resulted in agreement on a high-level process that Tapestry Networks developed in consultation with pilot participants. The pilots will thus provide a multi-stakeholder early-advice process that includes input and participation by regulators, HTAs, payers, patient advocates and clinical experts. All participating institutions will take part in discussions of therapeutic value, and a narrower group of HTAs and payers will consider and advise on questions of economic value. A sponsor who participates in a pilot will not lose the opportunity to utilise existing advice processes run by individual institutions.

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Institutional participants at the launch meeting expressed an interest in using the pilots to explore how close they can come to alignment on evidentiary requirements with their counterparts in other roles (i.e., payers and regulators) and across Member States, while maintaining their distinct responsibilities. Alignment would focus on what data sponsors should generate, with each Member State retaining its own approach to how it interprets the data. Participants also considered the pilots as an opportunity to identify areas where such alignment is not possible along with the reasons for the remaining differences.

Participants identified factors contributing to the pilots' success

Participants considered what success would mean for the pilots. They agreed that the pilots would be successful if they created a basis of experience and knowledge on which broader initiatives for early consultation could be built. Participants also expressed hope that the pilots could help identify commonalities and differences among stakeholders in how they approach value assessment of new medicines and, potentially, help to reduce the differences in the data that sponsors need to generate as inputs to those assessments.

Factors identified as critical to the success of the pilots include: setting and maintaining clear goals, working with open-minded participants with the appropriate expertise and ensuring a fair balance among stakeholders. Several called for continued effective management of the process by a neutral third-party as well as the commitment of sufficient resources by participating institutions.

Finally, meeting participants identified additional pilot details that need to be addressed in the near term. These include a mechanism for ensuring the confidentiality of commercially sensitive information provided by medicine sponsors; a communications strategy to clarify the pilot process for non-participants; determination of whether participants will provide advice to sponsors in their institutional or personal capacity (with a preference for institutional advice); addressing institutional resourcing needs to support participation; and consideration of how patient groups might make the greatest contribution to the pilots. Tapestry Networks will be following up on these issues as a prelude to medicine-specific consultations.

The meeting concluded with commitments by launch participants to move forward with the medicine-specific consultations and expressions of optimism for the success of the initiative.

Agreement on the need to pilot innovative an process for evaluating value in new medicines

Launch meeting participants brought to this initiative a shared sense that the current model of drug development is not meeting the needs of European healthcare systems effectively. There are a significant number of discrete evaluation procedures for new medicines across the EU, creating fragmented drug development requirements and different market access outcomes across Member States. Creating a collaborative process across stakeholders to provide better alignment on measures of value would potentially limit late-stage failures and lead to more efficient and effective medicinal research. This, in turn, would help drive down costs to the healthcare system while increasing the benefits flowing from the research being conducted.

Challenges within the drug development system to improving patient health outcomes

Participants identified a number of challenges with the current drug development system that make it difficult for healthcare systems to deliver the right drugs to the right patients at the right time. Participants agreed that enhanced early-stage, multi-stakeholder consultations present a meaningful opportunity to improve the drug development process and resulting health outcomes.

Specific challenges that participants expect the pilots to address include:

- **The high cost of bringing innovative medicines to market and the difficulty of funding effective new treatments.** As a senior regulator pointed out: *“We spend approximately \$60 billion every year to put on the market five or six new molecules that are first-in-class, which, by the way, does not mean that they represent true innovation. That means that the cost of drug development is absolutely terrific and we have to do something about that.”* An HTA leader underscored this need by noting that, under current financial constraints, *“effective new agents are appearing that we are unable to fund.”*
- **The development of drugs with little incremental benefit over existing treatments.** A medical expert pointed out that *“the cost of drug development today is not reasonable because of the small incremental benefit of many new drugs.”* According to a patient advocate, progress requires *“getting the real high-value products on the market, instead of just products that provide a very minor improvement.”*
- **The delay in getting newly developed high-value treatments to patients.** According to a medical expert: *“It has taken an enormous length of time to get the good treatments that we have got into patients, and that process I hope will be shortened and made more accurate.”* This delay is due, in part, to the frequent lack of adequate data for value assessment, as companies focus *“narrowly on the acquisition of data for the purpose of obtaining regulatory approval.”*

The promise of multi-stakeholder consultations in early-stage drug development

Participants believe that early-stage, multi-stakeholder consultations can help to address these challenges by focusing development programmes and aligning expectations across stakeholders regarding how the value of medicines should be demonstrated and assessed. In the words of a regulator: *“I think early-stage consultations can be helpful in defining the common set of objectives ... to avoid unnecessary surprises and wastage in the development programme such that, at the time of regulatory authorisation, relevant data has been generated for health technology assessment as well.”* Such alignment on the development plan also *“will increase the probability of success in confirmatory trials [by facilitating agreement on parameters such as] the right dose and the right target population.”*

HTA leaders recognised that the pilots represent a necessary increase in transparency by their institutions: *“We have the responsibility as representatives of society to help industry choose what society really needs and what health systems will value ten years from now.”* A fellow

HTA leader added that progress *“is a matter of being transparent on the tools that are being used to assess the value of medicines,”* while *“so far, one must confess that health technology assessment bodies and reimbursement bodies have not been very transparent about the methods they use.”*

Participants predicted that greater openness, along with a multi-stakeholder perspective and approach, would *“help companies understand what they need to measure to actually show value, either by the time of licensing or within the years closely thereafter.”* And, as noted by a regulator, the dialogue entailed by the pilots would help all stakeholders to *“understand if such requirements are doable.”*

Participants recognised that the pilots could even serve to *“nudge the key players in Europe towards greater harmonisation and alignment of our thinking about the evidential requirements for drug development.”* In their view, this initiative will provide greater clarity on three issues: first, what all parties can immediately agree in terms of methodology and evidence basis for a medicine; second, what parties can agree through dialogue; and third, where distinct differences remain sharply defined as unique national requirements. As an HTA participant concluded: *“If we are truly wanting some agreement or some understanding of what we can agree on and what we cannot, then having a joint process seems to be very important.”*

An industry participant concluded that *“we have created a common vision about what could be possible to make the drug development process more efficient and to align how we bring innovative medicines to patients. The pilots are about making sure we can deliver in this rapidly changing environment. Making the right decisions for patients is even more critical today.”*

Agreement on pilot design principles

The recommended pilot design was informed by learning from existing early consultation models, such as the EMA’s Scientific Advice Working Party, the UK National Institute for Health & Clinical Excellence (NICE) scientific advisory process, the new joint early advice process of the Swedish Dental and Pharmaceutical Benefits Agency (TLV) and Medical Products Agency (MPA), and the US Food and Drug Administration (FDA) early advice process. Tapestry Networks interviewed participants in these early advice processes to identify what aspects of their design worked well and what aspects could be improved.¹ The resulting initial design was further informed by extensive discussion at the launch meeting. [Appendix 3 provides additional details of several of the most widely utilised early advice processes.](#)

Participants at the launch meeting agreed the following six principles to guide the design and conduct of the pilots:

- **Agree to non-binding outcomes** to increase willingness to experiment, but with an expectation of *“engagement and commitment”* from the parties befitting the importance of this initiative. Additionally, advice should not displace or supersede existing channels for regulatory and reimbursement approval.

¹ Note that Working Group and industry participants applauded the spirit of innovation that led the MPA, the TLV and NICE to pilot early advice processes.

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- **Focus on methodology of value assessment** (as opposed to an early read on the potential for reimbursement or pricing). This is a common area within all parties' mandates, with therapeutic value informing the assessment of economic value in organisations with a health economic brief. However, given the differences across healthcare systems, economic evaluation shall remain uniquely the remit of Member States.
- **Foster open dialogue and equal standing** for all parties in discussion. As one HTA participant noted: *"We welcome the possibility of having different views from different countries and different perspectives, encouraged and advanced through the work of Tapestry."* The success of the pilots requires that all parties participate in good faith, as *"the improvement [in outcomes] will depend on the fact that everyone will be fair and engaged."* Such an environment cannot simply be mandated; however, it can be encouraged to develop. Experience with existing early advice processes suggests that a *"safe environment"* is most likely to be achieved when all parties participate actively, openly and collaboratively. For this reason, the pilots are designed to encourage *"regulators and HTAs ... to pose questions to industry and each other so that everyone could understand any concerns associated with a new compound."* Experience suggests that *"when regulators, HTAs and payers actively participate in this conversation, they can bring fantastic ideas."*
- **Create an environment that supports mutual learning.** There needs to be recognition throughout the pilots that this is an innovative process which can later inform other early advice processes and the drug development system. In the words of a clinical expert: *"We should listen and learn from the pilot process so that, in the future, we may have a more optimised way of arranging national and regional drug development structures."*
- **Encourage transparency** by publishing findings and keeping the process and participation in the pilots as transparent as possible, while keeping advice specific to a company's medicine confidential (as is customary).
- **Ensure that multiple Member States participate.** While some participants have suggested that the multi-Member State approach is *"quite ambitious,"* others acknowledged the need to reduce the complexity of navigating different requirements across health systems. According to such a view: *"It is very important that this effort doesn't become part of the fragmentation problem, [since] running a distinct process in every single Member State just contributes to more fragmentation of guidance and value indicators."* The multi-party interactions will, therefore, focus on implementation across a selection of five Member States, while acknowledging that their approaches to value assessment are distinct.²

Implicit in this approach is the recognition that the involvement of Network member companies is a stepping stone to broader participation, leading to *"an open process for others who want to join."* For example, this process would be open to additional EU Member State healthcare organisations who wish to participate. Similarly, several participants have suggested that it will be important to engage other pharmaceutical companies to help accelerate the learning arising from this process.

² The participating Member States for the pilots include France, The Netherlands, Sweden, the United Kingdom and Italy.

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Agreement on a high-level process for the pilots

At the launch meeting, Tapestry Networks put forth a proposed multi-stakeholder consultation process developed with the involvement of pilot participants, and informed by experiences with the early advice processes of NICE in the UK, Sweden's TLV-MPA and the EMA. The multi-stakeholder process is consistent with the design principles described in the previous section.

The consultations will be conducted recognising the centralised role for regulators in the disease areas chosen (breast cancer and type 2 diabetes), with the additional and equal participation of HTAs, payers, patient advocates and clinical experts. The agreed process creates an opportunity for both concurrent advice on issues of therapeutic value, and consultation with a narrower group of HTAs and payers on questions of economic value. Advice from all participants will be non-binding and participation in a pilot will not take the place of participation in other advice processes.

Overview of the pilot process

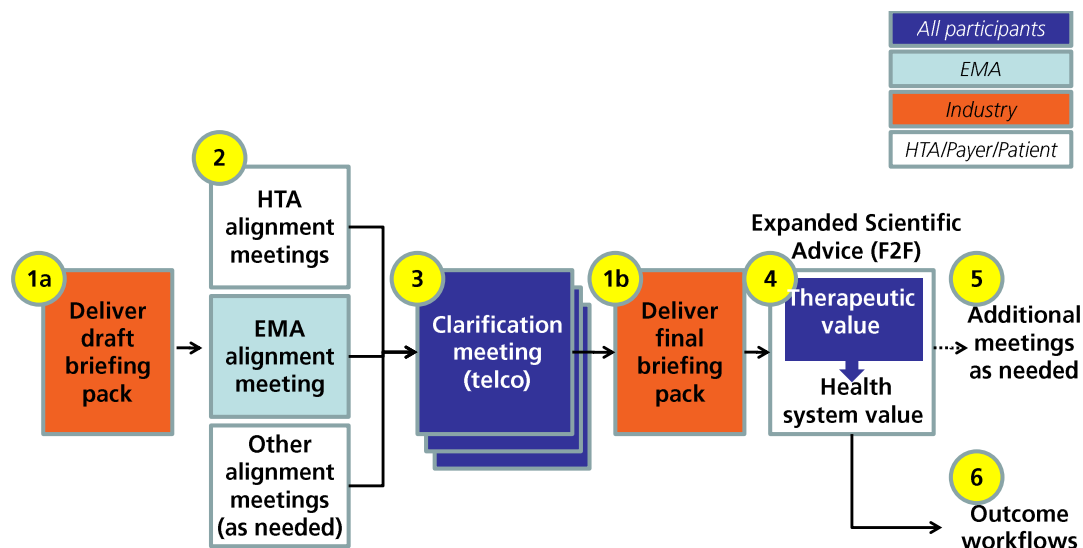
The launch meeting resulted in agreement on a multi-stakeholder consultation process that includes input and participation by regulators, HTAs, payers, patient advocates and clinical experts. All participating institutions will weigh in on issues of therapeutic value, and a narrower group of HTAs and payers will consider and advise on questions of economic value that flow from the medicine's therapeutic benefits. Launch meeting participants expressed interest in the possibility of reaching a common perspective across institutions regarding a medicine's therapeutic value, along with a "*mutual understanding of where we differ and why we differ.*" They recognised, however, that economic value determinations are solely the remit of Member States.

Meeting chairs for each part of the pilot meeting will be identified with specific expertise relevant to therapeutic or economic value. There was agreement among participants that the focus will be on capturing the methodology of value demonstration without seeking commitments on pricing or reimbursement from individual participating Member States. The Tapestry Networks team will co-ordinate meeting preparation, serve as process moderators during meetings and distil findings to support the potential broad roll-out of such consultations in the future. The graphic overleaf captures a high-level summary of the resulting process for multi-stakeholder consultations in early-stage drug development.

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Overview of multi-stakeholder consultations process in early-stage drug development



The agreed process creates an opportunity for concurrent advice on issues of both therapeutic value (with all stakeholders involved) and economic value (with a narrower group of HTAs and payers). The process will commence with a written request from a pharmaceutical manufacturer to the EMA and participating HTAs, payers, patient representatives and medical experts. The pharmaceutical company seeking advice will distribute a briefing pack (*step 1a*) with relevant available data and key questions pertinent to the value and development path of a specific medicine in order to focus the upcoming early advice consultation. This will be followed by internal meetings (*step 2*) within each participating institution if needed to clarify or modify industry questions and align on institutional feedback on the briefing pack in advance of the clarification meeting.

A series of multi-institutional teleconferences (*step 3*) will inform any updates to the briefing pack to reflect participants' questions on scope and content. There was significant interest amongst several participants to align on a common set of questions in order to access advice that is *"informed by common perspectives"* while still *"producing individual institutional advice."*

After distribution of the revised briefing pack (*step 1b*), a half-day face-to-face consultation will be held (*step 4*). During this session, advice and discussion will be provided concurrently by regulators, HTAs and payers, and will include expert clinical and patient representative inputs to industry. As discussed above, the end goal for this advice process is to clarify the value profile of the medicine and articulate how that value should be demonstrated and assessed.

Participants representing healthcare institutions expressed interest in discussing the advice they plan to give to industry with other participating institutions, so as to *"gradually get to greater understanding of each institution's perspective"* in order to *"foster a dialogue about where each participant agrees, where they might agree with a bit of conversation and where they agree to disagree."*

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The pilot process provides an option to convene additional advice meetings (**step 5**) as needed, in recognition that *“drug development is a dynamic process and science evolves.”* This mechanism would provide participants *“the opportunity of looking at things again as information evolves.”* At the end of the pilot phase, Tapestry Networks – in partnership with pilot participants – will distil key lessons (**step 6**) to inform the design of subsequent pilots and other current or future multi-institutional advice processes.

The agreed approach places the primary responsibility on industry for capturing the content of the discussions. In addition, participating institutions will follow their customary practice, be it formal or informal, in rendering advice. For example, advice by EMA as to therapeutic value will be provided in written form after vetting by the agency’s Scientific Advice coordinators with the Committee for Medicinal Products for Human Use (CHMP) in accordance with the EMA’s Scientific Advice process. Regardless of how participating institutions contribute to the discussion, all advice will be non-binding. The consultation process is expected to take 60 days from the delivery of the draft briefing pack to the face-to-face consultation meeting.

Launch meeting participants acknowledged that the circumstances of individual medicines for which consultation is sought may warrant a variation on the outlined design, in line with the experimental nature of this initiative.

An opportunity to align evidence requirements

Participants are looking to the pilot process to support, in the words of an HTA leader, getting as *“close to alignment as possible”* across stakeholders. As a regulator agreed: *“We have two thoughts in the room now: one is, we are trying to align, we are trying to address the same questions and to get to unified, harmonised answers from the different players; on the other hand, we have to think about our different responsibilities. I believe the process outlined has taken great care to make sure those different areas of responsibility are preserved.”* Additionally, participants noted that inclusion in the pilot process of the FDA, a key regulatory player in shaping medicinal development, would further the goal of aligning evidence requirements for the value demonstration of new drugs.

Participants generally supported this pilot approach as one that could be reconciled with their existing processes. As summarised by an HTA participant: *“This pilot process [of involving a broader group of stakeholders] is not such a big step for us. It does not feel too much of a burden if we can incorporate it into our existing structure.”* Beyond these pilots, there is a uniform recognition among participants that any process developed would eventually need to be institutionalised in a manner that fits within each participating organisation’s mission and operating model. In the near term, the pilots would serve, as noted by a regulator, as *“a platform from which we can all build ... This looks like a promising way forward.”*

Factors contributing to the success of the pilots

Looking beyond process details, participants considered what would define success for the pilots. Desired outcomes include an initiative that *“develops a successful process for multi-stakeholder consultation ... that could be adapted to other products and disease areas,”* thereby providing a

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“model for subsequent pilots.” Moreover, this process should provide an *“understanding of common areas among participant stakeholders and of where the gaps/differences remain,”* and should give rise to *“advice that informs drug development”* with a clear impact on decisions made by industry to shape development plans. Finally, participants considered the processes of *“learning across pilots and sharing that learning”* to be of utmost importance to successful outcomes from this initiative. Tapestry Networks will work with pilot participants to assess the performance of the pilots along these measures of success.

There was general consensus among participants as to the critical factors required to support these desired successful outcomes – including *“clear goals,”* the *“open-mindedness of participants”* and having the *“right experts present”* to provide the insights required – while making sure that a *“fair balance is maintained in discussions across stakeholders.”* Several participants called for the continued *“excellent management of process”* to make these outcomes possible. Finally, all agreed that these objectives need to be supported by the *“commitment of sufficient resources from all participants.”*

Launch meeting participants also identified issues that remain to be addressed for the success of the pilots. These include managing the confidentiality of medicine-specific data, communicating proactively toward non-participants about the pilot process, ensuring the clarity of stakeholder roles and addressing institutional resourcing needs to support participation. At the time of this writing, the Tapestry Networks team is engaged with participating institutions to address these issues.

Confidentiality

Participants recognised that the content of briefing packs provided by companies seeking multi-stakeholder advice is likely to contain elements that constitute confidential business information. While regulators have experience managing confidentiality in the course of rendering scientific advice, other participants may have less experience managing confidential information. Participants believe this issue can be addressed through the use of confidentiality agreements. A regulator noted that, *“given our experience, we do not think that legal or confidentiality problems are difficult to resolve.”* To streamline this aspect of the process, Tapestry Networks has developed and validated a single confidentiality agreement for use across the pilots.

Need for proactive communication to support transparency

A number of participants voiced the need for clear and directed communications about the pilots to prevent the risk of non-participants misinterpreting this initiative. An HTA advisor noted that *“our work here is very new, very innovative, but not communicated well ... we should communicate the playing rules for others who might wish to participate. We should describe this on the internet or via other accessible channels.”* To this end, Tapestry Networks is working with pilot participants to determine and carry out an appropriate communications strategy. As a starting point, Tapestry has posted on its website an updated overview of the pilot process and the initiatives leading up to it.³

³ Information on the pilot process can be found at [“Creating a more sustainable model for innovation in an era of scarce resources.”](#)

Creating conditions for institutional participation

Participants agreed that the pilots (and industry, as the seeker of advice) would be best served through institutional advice rather than the individual opinions of institutional personnel. Several participants, including the EMA, HTAs and payers have committed to providing advice in such an institutional capacity. An HTA participant, however, noted that engaging at the institutional level presents implications for resourcing and logistics: *“The closer the advice gets to being issued from an institutional perspective, the more bureaucratic and resource-intensive it will be ... We would like to take the approach of providing institutional advice, but with that there are resource consequences.”* On the basis of this input, Tapestry Networks is designing a resourcing model to support the pilots.

The role of patient groups in the pilot process

The pilots will provide an opportunity for learning about the nature of the contribution patients can provide to a consultation at such an early stage of drug development. A regulator noted that *“we have used patients in advice and it has been fruitful and effective ... it is going to be a challenge, but an interesting one.”* Acknowledging the challenge, one patient representative suggested that *“additional education could be required upfront for patient advocates who will contribute to the process.”* Consequently, Tapestry Networks is exploring a framework wherein patients may be sufficiently informed about scientific and process issues to participate effectively, while maintaining an independent patient perspective. A potential solution is to provide clinical expertise directly to the patients to help educate them on the technical issues and work with the patient groups to provide *“the lightest yet most impactful process to provide the fewest barriers for progress in the pilots.”*

Schedule of pilot consultations

Tapestry Networks is working with industry medicine development teams to prepare briefing books and timelines, with a view to scheduling the first consultation in type 2 diabetes for 25 October 2010, the second consultation in type 2 diabetes for 2 December 2010, and a breast cancer pilot consultation for December 2010–January 2011.

Conclusion

On 14–15 July 2010, healthcare leaders representing institutions from five Member States and the EMA gathered in London to launch pilots of multi-stakeholder consultations in early-stage drug development. They did so amid growing acceptance across Europe that by overcoming barriers to collaboration and aligning on value, real progress can be made to address the rising cost of medicines and the declining rate of innovation. The pilots represent an experimental process to jointly design a new drug development approach based on the input of multiple stakeholders.

The launch meeting was attended by pragmatic, open-minded and courageous individuals representing organisations that are willing to look beyond their traditional boundaries for solutions. Participants expressed a strong commitment to moving forward on the pilots while acknowledging that the challenges involved can only be addressed through persistence and

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collaboration. A clinical expert voiced the general consensus of the group on the pilot's aims, stating that *“everybody here has the same objective, the same goal, and we are not so far from one country to another.”* Achieving those aims will require participants to *“be patient and help each other along the way,”* as an HTA leader candidly noted, *“because I realise there is a problem and I do not know how to solve it alone.”*

The meeting ended with a shared sense of optimism that collaboration and shared perspectives can achieve the goal of improving the effectiveness of the drug development process in delivering innovative medicines that address unmet patient needs at reasonable cost. As an HTA leader summed up: *“I am optimistic because we have something tangible here. There are lots of change agents in this room. When we have a few change agents in a room of people who do not want to change then nothing much happens. When you have lots of change agents in the room who knows what is going to happen.”*

About this document

The views expressed in this document represent those of individuals affiliated with the European Healthcare Innovation Leadership Network, the two disease-specific Working Groups it convenes, and other healthcare leaders whose insight has shaped the development of this initiative. This document is not intended to represent the policies or positions of any particular individual, institution or organisation. This material is prepared by, and the copyright of, Tapestry Networks. It may be reproduced and redistributed, but only in its entirety, including all copyright and trademark legends.

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Appendix 1: list of institutions contributing to the pilots

Institutional participants
France
Agence Française de Sécurité Sanitaire des Produits de Santé (AFSSAPS) Comité Economique des Produits de Santé (CEPS) Haute autorité de santé (HAS) Transparency Commission
Italy
Agenzia Italiana del Farmaco (AIFA) Italian Medicines Agency
The Netherlands
CVZ Dutch Diabetes Association Netherlands Breast Cancer Association (BVN) Menzis UVIT
Sweden
Swedish Medical Products Agency (MPA) SKL Landsting County Councils Swedish Breast Cancer Association (BRO) Dental and Pharmaceutical Benefits Agency (TLV)
United Kingdom
Medicines and Healthcare products Regulatory Agency (MHRA) National Institute for Clinical Health and Excellence (NICE) National Health Service Primary Care Trusts (Derbyshire County, Redcar & Cleveland, Stockton-on-Tees)
Europe
European Medicines Agency EUnetHTA (<i>Observer</i>) EUROPA DONNA (<i>Observer</i>)
United States
The Food and Drug Administration (<i>Liaison</i>)
Industry
AstraZeneca GlaxoSmithKline Johnson & Johnson

Appendix 2: overview of the European Healthcare Innovation Leadership Network and the road to the pilots

Creating a more sustainable model for innovation in an era of scarce resources

The European Healthcare Innovation Leadership Network was established by Tapestry Networks in 2006 with the support of GlaxoSmithKline and Andrew Witty. GSK, AstraZeneca and Johnson & Johnson now support and fund this independently led initiative. The Network brings together a premier group of healthcare leaders from EU Member States who are committed to addressing the complementary goals of improving patient health outcomes as well as the climate for innovation within the constraints of pressures to control healthcare costs. *(Please see attached list of Network members).*

Network members are motivated by a confluence of factors: we are entering a “*new world*” in which generics are more readily available and represent an economically attractive alternative to innovative medicines. Meanwhile, R&D is “*in a period of low productivity*” and all stakeholders face increasing economic pressures. While progress on chronic diseases and a scarcity of resources have sharpened the focus on assessing and demonstrating the value of new medicines, continuing to address unmet medical needs and improve patient outcomes remains the ultimate goal for all stakeholders. A fundamental component of supporting this goal is the development of more differentiated market access, pricing and reimbursement solutions to reflect – and reward – the actual health and economic value delivered by different medicines. This, in turn, requires a “*Shared Value Framework*” – a common framework and approach for how such value can be defined, assessed and demonstrated for a given medicine.⁴

Creating Shared Value Frameworks for evaluating new medicines

Network members recognised that moving the value discussion from the abstract to concrete outcomes required a disease-specific focus. Network members and Tapestry Networks identified two initial focus areas chosen for their high unmet needs and impact on healthcare systems – type 2 diabetes and breast cancer; the former presents a chronic progressive illness with an increasing demographic and significant societal impact and the latter presents significant cost burden of medicines to healthcare budgets. In 2009, initiated by the Network, Tapestry Networks convened the type 2 diabetes and breast cancer Working Groups. The Working Groups consisted of leading medical experts, regulators, payers, reimbursement authorities, patients and industry representatives across EU member states *(Please see the list of Working Group participants on the following pages).*

Working together over the course of 2009, Working Group participants established a Shared Value Framework – an agreed set of attributes, therapeutic endpoints and economic inputs – for evaluating new medicines within specific therapeutic areas. The participants additionally recommended piloting a new collaborative approach across stakeholders to apply such a

⁴ The development and significance of the Shared Value Framework concept is described in [“Developing shared value frameworks and setting the stage for new forms of collaboration among stakeholders.”](#) *ViewPoints*, 3 December 2007.

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framework to real world medicines under development and generated initial design principles informed by the open working culture of the groups.⁵

Working Group participants believe that piloting multi-stakeholder consultations in early-stage drug development is potentially a rich source of value, including:

- Better allocation of scarce resources for industry, payers and regulators
- Deeper insight into the pipeline of new medicines for payers, physicians and patients to provide better data earlier for more informed planning and decision-making
- Stakeholder alignment on the specific pre- and post-launch activities needed to assess and demonstrate the value of a new medicine
- Earlier development of clinical practice guidelines for patients and providers to help ensure that value is realised

At the Network's January 2010 meeting, the Network affirmed the Working Groups' recommendations and, with the support of Tapestry Networks, catalysed the launch of pilots of multi-stakeholder consultations in early-stage drug development to apply and test the Shared Value Frameworks. Network members recognise that no single stakeholder group acting alone can successfully accomplish this goal. Thus, the call went out in support of the pilots, *"to demonstrate how you might design something different where all stakeholders and patients would benefit."*

⁵ For summaries of the Working Groups' recommendations, see ["Improving Health Outcomes in Breast Cancer: Recommendations of the Breast Cancer Working Group"](#) and ["Improving Health Outcomes in Type 2 Diabetes: Recommendations of the Type 2 Diabetes Working Group."](#)

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Members

Member States

Czech Republic

- **Pavel Hroboň** | Former Deputy Minister | Ministry of Health

France

- **Eric Abadie** | Direction Générale | Agence Française de Sécurité Sanitaire des Produits de Santé (AFSSAPS)
- **Noël Renaudin** | President | Economic Committee for Health Products (CEPS)

Germany

- **Rainer Hess** | Impartial Chairman | Federal Joint Committee (G-BA)
- **Wolfgang Schmeinck** | Beauftragter des Vorstandes | BKK Landesverband Nordrhein-Westfalen

The Netherlands

- **Mike Leers** | Advisor – Board of Commissioners | CZ Healthcare Insurance Group
- **Martin van Rijn** | CEO | PGGM

United Kingdom

- **Mike Farrar CBE** | Chief Executive | National Health Service – North West
- **Sir Michael Rawlins** | Chairman | National Institute for Health and Clinical Excellence (NICE)
- **Professor Sir Mike Richards CBE** | National Clinical Director for Cancer & End of Life Care | St Thomas' Hospital

Pharmaceutical Innovators

- **Eddie Gray** | President, Pharmaceuticals Europe | GlaxoSmithKline
- **David Norton** | Company Group Chairman – Global Pharmaceuticals | Johnson & Johnson
- **Ulf Säther** | Regional Vice President, Europe | AstraZeneca

Other Key Constituents

- **David Byrne** | Former EU Commissioner – Health and Consumer Protection
- **Thomas Lönngren** | Executive Director | European Medicines Agency (EMA)
- **Anders Olauson** | President | European Patients' Forum
- **Sophia Tickell** | Non-Executive Director | Sustainability & Director | Pharma Futures

Breast Cancer

Medical subject matter experts

- **Jonas Bergh**, Karolinska Institute, Sweden
- **PierFranco Conte**, Universitaria di Modena, Italy
- **Jindřich Fínek**, University Hospital Plzen, Czech Republic
- **Luca Gianni**, University of Milan, Italy
- **Anthony Howell**, The Christie NHS Foundation Trust, UK
- **Christian Jackisch**, Klinikum Offenbach GmbH, Germany
- **David Khayat**, Pitié-Salpêtrière Hospital, France
- **Jan Lubiński**, Pomeranian Medical University, Poland
- **Larry Norton**, Memorial Sloan-Kettering Cancer Center, USA
- **John Robertson**, University of Nottingham, UK
- **Karol Sikora**, CancerPartners UK, UK
- **Michael Untch**, HELIOS Klinikum, Germany

Payers, regulators, health economists and advisors

- **Johannes Bruns**, Deutsche Krebsgesellschaft, Germany
- **Karl Claxton**, University of York, UK
- **Pierre Démolis**, Agence Française de Sécurité Sanitaire des Produits de Santé (AFSSAPS), France
- **Harald Enzmann**, Bundesinstitut für Arzneimittel und Medizinprodukte (BfArM), Germany
- **Pavel Hroboň**, formerly Ministry of Health, Czech Republic
- **Bengt Jönsson**, Stockholm School of Economics, Sweden
- **Bertil Jonsson**, Medical Products Agency, Sweden
- **Sören Olofsson**, Region Skåne, Sweden

Patient representatives

- **Els Borst-Eilers**, Dutch Federation of Cancer Patients, The Netherlands
- **Susan Knox**, EUROPA DONNA, European Breast Cancer Coalition (*Observer*)

Industry representatives

- **Jim Baker**, Johnson & Johnson
- **Alan Barge**, AstraZeneca
- **Paolo Paoletti**, GlaxoSmithKline

Diabetes

Medical subject matter experts

- **Amanda Adler**, Institute of Metabolic Science, Cambridge, UK
- **Jean-François Bergmann**, Hôpital Lariboisière Paris, France
- **Christian Berne**, Uppsala University, Sweden
- **Bernard Charbonnel**, University of Nantes, France
- **Ele Ferrannini**, University of Pisa School of Medicine, Italy
- **Vivian Fonseca**, Tulane University Medical Center, USA
- **Philip Home**, Newcastle University, UK
- **Harald Klein**, Ruhr-Universität Bochum, Germany
- **Mohan Kumar**, NHS North Western Deanery, UK
- **Andrew Morris**, University of Dundee, UK
- **Eberhard Standl**, Munich Diabetes Research Institute, Germany

Payers, regulators, health economists and advisors

- **Andrew Briggs**, University of Glasgow, UK
- **Hans-Georg Eichler**, European Medicines Agency (EMA)
- **Peter Kolominsky-Rabas**, University of Erlangen-Nuremberg, Germany
- **Félix Lobo-Aleu**, Universidad Carlos III, Spain
- **Noël Renaudin**, Economic Committee for Health Products (CEPS), France
- **Michael Schlander**, Institute for Innovation and Valuation in Health Care, Germany
- **Sjaak Verduijn**, CZ Insurance, The Netherlands

Patient representatives

- **Maarten Ploeg**, Dutch Diabetes Association, The Netherlands

Industry representatives

- **Martin Fitchet**, Johnson & Johnson
- **Gunnar Olsson**, AstraZeneca
- **Carlo Russo**, GlaxoSmithKline

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PILOTS OF EARLY-STAGE MULTI-STAKEHOLDER CONSULTATIONS

Appendix 3: overview of selected early advice processes

Sponsor	Process name	Consultation format	Duration	Meeting chair	Participation	Process time (initial request to final advice)
EMA	Scientific Advice Working Party (SAWP)	Written / Face to Face ¹	~90 min	2 EMA coordinators with specialised clinical expertise	<ul style="list-style-type: none"> SAWP appointees Company representatives Invited company guests 	100–130 days
FDA (PDUFA)	Special Protocol Assessments	Face to Face	~90 min	FDA coordinator (generalist assigned by therapeutic area)	<ul style="list-style-type: none"> FDA Company representatives Expert consultants 	45–95 days ²
MHRA	Scientific advice for license applicants	Face to Face	~90 min	MHRA coordinator (generalist assigned by therapeutic area)	<ul style="list-style-type: none"> MHRA Company representatives 	60–120 days
NICE	Scientific advice	Face to Face	180–240 min	NICE coordinator (generalist assigned by therapeutic area)	<ul style="list-style-type: none"> NICE appraisal committee Company representatives Expert NICE consultants 	100–150 days
TLV /MPA	Joint Scientific Advice	Face to Face	~90 min	MPA coordinators	<ul style="list-style-type: none"> MPA and TLV Company representatives 	Variable

¹ The SAWP process begins with an initial meeting between the company and the coordinator(s) and/or the coordinator's experts and the EMA secretariat. The SAWP multidisciplinary panel may also invite the company to meet face-to-face at a subsequent SAWP meeting discussion meeting.

² This time includes 6-8 weeks needed to vet consultants for external conflicts prior to consultation.

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PILOTS OF EARLY-STAGE MULTI-STAKEHOLDER CONSULTATIONS

Appendix 3: overview of selected early advice processes (continued)

Sponsor	Focus of early consultation	Timing of early consultation	How advice is delivered	Is advice binding?	Consults / year
EMA (SAWP)	Planning confirmatory trials (Clinical safety and efficacy, e.g. pharmacovigilance, risk management and pre or post-authorisation activities)	Phase I, II or III	Written format ¹	No ²	~310/ year
FDA (PDUFA)	Planning confirmatory trials (Clinical protocols for phase III trials whose data will form the primary basis for an efficacy)	End of phase II ³	Written format	Yes	~350/ year ⁴
MHRA	Planning confirmatory trials (Clinical endpoints, trial duration, target population, choice of comparator, etc.)	Phase I, II or III	Written format	No	~250/ year
NICE	Planning confirmatory trials and developing health economics rationale (Clinical efficacy and cost effectiveness)	Flexible, typically end of phase II	Written format	No	~35/year
TLV / MPA	Planning confirmatory trials and developing health economics rationale (Clinical efficacy and cost effectiveness)	End of phase II	Verbally ⁵	No	~10/ year (currently)

¹ Minutes are considered to be a company's record of the meeting and will not be endorsed by the SAWP.

² If companies choose not to apply Scientific Advice, they are requested to justify clearly their position in any subsequent application for marketing authorisation.

³ FDA Special Protocol Assistance can also be sought for label extensions of authorised products.

⁴ In 2008 the FDA engaged in 354 Special Protocol Assessments, the most recent figure available. The five year average from 2003-2007 is 380.

⁵ In addition to the face to face discussion, the company is responsible for taking minutes which may serve as a record of the advice. Neither the TLV nor the MPA will officially sign off on these minutes.