Value for money in pharmaceuticals: fostering constructive collaboration among stakeholders

Introduction

The European Healthcare Innovation Leadership Network convened for its second meeting on 1-2 March 2007. The Network comprises decision-makers from Member State health, industry and finance ministries, the pharmaceutical industry and other key constituencies. The purpose of the Network is to facilitate collaboration, generate insights and formulate solutions for achieving health objectives and reinvigorating pharmaceutical industry competitiveness. At the inaugural meeting, members generally agreed that current interpretations of innovation in medicines were inadequate for assessing value, and that identification of value criteria was required. Although members converged on the idea that value in pharmaceuticals might best be described as health outcomes for patients, they agreed that further consideration was required regarding how value is assessed, measured and aligned with price. Thus, the overarching objectives of the second meeting were to advance collective understanding of value for money in pharmaceuticals and consider risk sharing models that might inspire new forms of collaboration among stakeholders.

The agenda for the meeting was developed based on interviews and meetings with members and subject matter experts from across Member States. Members identified three principal topics on which to focus during the second meeting. First, they debated sources of value described in a spectrum ranging from therapeutic outcomes to broader societal outcomes. Next, they discussed the requirements for fully assessing, measuring and capturing value from innovative medicines, and finally, they shared perspectives on risk sharing arrangements designed to better align interests among stakeholders. They concluded by establishing priorities for future collaboration.

Members who attended the meeting were:

- Bruno Angelici, Executive Vice President, Europe, Japan, Asia-Pacific, Latin America, AstraZeneca
- Monica Bettoni Brandani, Head of the Technical Secretariat of the Minister, Ministry of Health, Italy
- Emmanuel Caquot, Head of Manufacturing Industries Service, Ministry of Economy, Finance and Industry, France
- Sir David Cooksey, Founder, Advent Venture Partners
- Pierre-Jean Lancry, Director, Caisse Centrale Mutualité Sociale Agricole, France
- Mike Leers, Chairman and CEO, CZ Healthcare Insurance Group
- David Y Norton, Company Group Chairman, Worldwide Commercial & Operations, Johnson & Johnson
- Anders Olauson, President, European Patients’ Forum
• María Luisa Poncela, Deputy Director-General of Regional Incentives, Ministry of Economy and Finance, Spain

• Sir Michael Rawlins, Chairman, National Institute for Health and Clinical Excellence (NICE), United Kingdom

• Noël Renaudin, President, Economic Committee for Health Products (CEPS), France

• Ann-Christin Tauberman, Director-General, Pharmaceuticals Benefits Board, Sweden

• Sophia Tickell, Chairperson, SustainAbility

• Martin van Rijn, Director-General, Healthcare, Ministry of Health, Welfare and Sport, Netherlands

• Andrew Witty, President, Pharmaceuticals Europe, GlaxoSmithKline

In addition, several members were unable to attend the meeting, but most participated in discussions with Tapestry Networks prior to the meeting. ViewPoints includes selected comments and perspectives from these members, as well as several additional subject matter experts:

• David Byrne, Pathwell Limited

• Rainer Hess, Impartial Chairman, Federal Joint Committee, Germany

• Pavel Hroboň, Deputy Minister, Ministry of Health, Czech Republic

• Franz Knieps, Director-General, Public Healthcare, Health Insurance, Long-term Care, Federal Ministry of Health, Germany

• Ignazio R. Marino, Chair, Health Committee, Senate of the Republic of Italy and Professor of Surgery, Jefferson Medical College

• Nello Martini, Director-General, Italian Medicines Agency (AIFA), Italy

• Wolfgang Schmeinck, CEO, National Federation of Company Sickness Funds, Germany

ViewPoints is a synthesis of key issues arising from discussions among members of the European Healthcare Innovation Leadership Network. It is produced by Tapestry Networks to stimulate timely, substantive discussions about the choices confronting key stakeholders from Member States and the pharmaceutical industry as they endeavour to fulfil their respective responsibilities related to the healthcare of Europe’s citizens and the policies for a vibrant R&D environment. ViewPoints reflects the Network’s use of a modified version of Chatham House Rule whereby names of members and their affiliations are a matter of public record, but comments made during meetings are not attributed to individuals or organisations. The ultimate value of ViewPoints lies in its power to help all constituencies develop their own informed points of view on these important issues. It may be shared freely.

1 Neither Mr Knieps nor Dr Martini was available to participate in pre-meeting discussions.
Executive summary

“Restlessness and discontent are the first necessities of progress”.
- Thomas Edison

Members came together in acknowledgement that the pharmaceutical industry and healthcare delivery in Europe “are at a crossroads” and “the economic future is perilous”. Members indicated that they were not only restless with the status quo but that a crisis might be imminent. One member noted, “Where there is a crisis, there is action” while another agreed, “Crisis leads to resolution. I hope healthcare doesn’t get to crisis before we have resolution”. Members recommitted themselves to working collaboratively to avert the crisis.

Network members generated insights centred on the following themes:

- **Vantage points on value assessment and meeting objectives (pages 4-5)**
  As they did at the last meeting, members asked “through whose eyes is value being assessed?” They accentuated the relevance of three sets of stakeholders depicted in Figure 1: “patients, industry and payers”.

- **Assessing value for money in pharmaceuticals (pages 6-11)**
  Members reflected on a spectrum of value (Figure 2, page 7) and identified measures of therapeutic, health system and societal value (Appendix 2). A conundrum emerged in that all members acknowledged the importance of societal value but also pointed to the inherent complexities in measuring and capturing it. Consequently, several members (primarily payers) asserted that focus should remain on therapeutic and health system value, while others advocated broader recognition of value.

- **Demonstrating value in the real world (pages 11-15)**
  Members highlighted the importance of assessing new drugs in ‘real world’ settings as a step toward “better aligning the price of drugs with their value to patients and society”. They noted the inadequacy of clinical trials, shortage of post-launch data and need for integrated data collection mechanisms as contributors to the misalignment between price and value.

- **Risk sharing arrangements as an example of value-based pricing (pages 15-22)**
  Members acknowledged that risk sharing is not an option for all situations and began to identify benefits, risks and conditions under which such arrangements might be appropriate. They stressed the need for effective contracting and noted that risk sharing agreements change the focus of negotiation from price to value. They also noted that more constructive collaboration among stakeholders is required to ensure success of value-based pricing models.

- **Forward moving agenda (pages 22-23)**
  Members agreed two work streams that they would collectively pursue in advance of the next meeting. They will continue to build upon the work conducted to date in developing shared value frameworks by identifying measures of value and assessment methodologies. They will also work collectively to develop a set of guidelines and conditions for pilots and experiments designed to assess, enhance and capture therapeutic, health system and societal value from medicines.
Vantage points on value assessment and meeting objectives

"If you want to truly understand something, try to change it."  – Kurt Lewin

A key question that continues to surface in discussions of value for money is “through whose eyes?” value should be assessed. One member highlighted three key sets of stakeholders that members consider in their evaluations of value when he asked: “Through the eyes of the patient, industry or the payer?” As members shared their personal objectives for the meeting, they referenced these vantage points on value assessment and indicated that striking a balance between the sometimes competing objectives of each set of constituents is one of the primary objectives of drug policy. A member had previously framed the tension: “The piece on the front burner is to strike an equitable balance between [industry] and the payer” while “maximising health outcomes for patients”. A payer reinforced the interconnection: “We are constantly looking for the best compromise between patients, industry and payers”.

Figure 1 illustrates these three vantage points on value assessment and the primary objective associated with each. A government member suggested that “some kind of triangle [of objectives] exists - first is patients [seeking health outcomes], second is promoting innovation, and third is cost control”. He emphasised each facet of the triangle in his opening comments positing that it was necessary to “try to give an answer to the question we have in all western governments of how to face rising costs” while “making it possible for the pharmaceutical industry to invest in innovation for the future” and providing value for patients. A fellow member highlighted two facets of the triangle in describing the role of drug policy: “We use reimbursement to get as many health outcomes for patients as possible and to stimulate industry to give us even more innovative pharmaceuticals”.

Figure 1 – Vantage points on value assessment

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2 To maintain anonymity of members, masculine forms of pronouns (ie, he, his) are used throughout this document.
Although the “triangle” admittedly discounts the complexities embedded in reimbursement decisions, its simplicity resonated with members. In conversation prior to the meeting, one member accentuated the challenges associated with accommodating industry, patient and payer objectives as he described “three constraints of developing drug policy”. He enumerated: “First, we must focus on facilitating innovation. Otherwise, we will have no new drugs and nothing else to discuss. We must work under the condition that we need progress. We cannot wait and act as if others will pay for innovation; we cannot act as free-riders. We have to act in such a way that if other nations behave similarly, we will all still be well off. Second, in a democracy, it is necessary that we provide the same level of healthcare and access to drugs to all our citizens. Third, what links the first two is that these have to be sustainable. It would be easy to ensure innovation and access to health if funding was endless. We have to develop a sustainable approach”. The three objectives of promoting innovation, providing health outcomes for patients and controlling costs are explicit in his explanation.

This same member went on to articulate the mechanisms for balancing these objectives, and in so doing, set up the theme of the second Network meeting: fostering collaboration to achieve value for money in pharmaceuticals. He said: “We have only two instruments for managing the constraints. One is to decide where we accept to pay more [for drugs]. The other is to make good use of them – to use drugs appropriately”. His comments reflected the realisation that it is critical to determine where the value lay in new drugs and to ensure proper administration to achieve value for money. Accomplishing this will require thoughtful consideration of the objectives of each set of stakeholders as well as collaboration among those stakeholders.

His fellow members supported this theme as they shared their objectives for the meeting. One member said he “wanted to find out other’s views of what value is” in medicines bearing in mind “the tension between possibility and affordability” in delivering them. Others expressed greater specificity; they were most interested in “how to define value” – “to get, step by step, to better mutual understanding of value and a methodology to reward it”. A member emphasised that “it’s difficult to establish any evaluation process without this. We have to have a common language about value”. Another added: “If stakeholders in Europe can achieve alignment with the pharmaceutical sector related to what value means for all of us, how value is created, and agreement of a common concept between administrations, patients and industry, then we are headed in the right direction for the future”. Yet another member alluded to an overarching objective shared by several members when he suggested he wanted to more deeply explore “how industry succeeds in getting drugs to people in different countries with different systems” and was most interested in “the contradictions that lay beneath the surface”. He asked: “In the real world, how do we make this work?”

A member summarised the preceding discussion when he shared: “We have a mixed constituency here: payers, patients, industry. Like everyone else, I am interested in value. I am not convinced that we will have a solution today – and it may not be one-size-fits-all because systems are different – but, if there are openness and willingness to work toward mutual understanding of value and to start some experiments, those are good first steps. They are first steps in a relatively long process”. Thus, members recognised that the journey upon which they were collectively embarking would be neither easy nor short; instead, it would require vigilant and constructive collaboration on each of their parts to ensure that progress was made.
Assessing value for money in pharmaceuticals

“What we obtain too cheap, we esteem too lightly; it is dearness only that gives everything its value”.

- Thomas Paine

At the first Network meeting, members agreed that value for money in pharmaceuticals could be described simply as health outcomes for patients measured over time in the real world. However, discussions with members in advance of this meeting revealed that the apparent consensus regarding the definition of value for money concealed a wealth of subtleties in the understanding of value itself. They said: “If we are talking about outcomes, everyone looks at it slightly differently” and “The same words often mean something different”. Furthermore, members emphasised how critical it was to link value assessments to the reimbursement process because “reimbursement is guided by explicit rules” that members suggest too often seem to be disconnected from measures of value.

In discussions with members and subject matter experts prior to the meeting, it became evident that reimbursement authorities consider a spectrum of value that, not surprisingly, industry participants believed was too narrow. One member conceded that “most countries offer too narrow a spectrum of value or benefit” and another added that “it’s time to broaden it”. One industry participant supported his argument for broader consideration of value by pointing to market structure: “In a monopsonistic market where governments, in effect, make the market, governments have a certain obligation to embrace a relatively broad inclusion of value drivers”.

Primary objectives of this meeting were to further refine member perspectives on the spectrum of value considered in reimbursement decisions and to explore possibilities to better align value with price.

In preparation for the meeting, members and subject matter experts reflected on how value in pharmaceuticals is assessed in their Member States and how shared frameworks might facilitate better alignment between value delivered and value received from innovative medicines. The following themes will be discussed in this section:

- **A spectrum of value exists for consideration in reimbursement decisions.** A spectrum of value emerged that encompasses therapeutic value, health system value and societal value. Member States focus differentially on each source of value.

- **Societal value presents a conundrum.** While acknowledging its importance, members lamented the limitations in measuring and capturing societal value. Members debated unemployment, carer considerations, and innovation as critical elements of societal value.

- **Measuring and capturing value becomes increasingly difficult with attempts to incorporate the wider spectrum of societal value.** Members noted deficiencies in health systems to accommodate the full spectrum of value and expressed disappointment in their existing ability to reconcile expenditures in one facet of the budget with savings or benefits in other budgets.
A spectrum of value exists for consideration in reimbursement decisions

Figure 2 illustrates the spectrum of value that emerged from conversations with members and subject matter experts in advance of the meeting. At its core is therapeutic value, onto which additional sources of value such as savings to the health system and broader economic and societal value can be added. The words of one industry member served to describe the spectrum of sources of value. He defined value for money of a new medicine simply: “A product that is going to offer better outcomes than the current situation”. He added: “These outcomes are varied and many. We should look at this from a holistic perspective. Outcomes include an improvement in overall patient well-being as well as reductions in overall health and societal costs”. He offered further specifics and in the process summarised the spectrum of value articulated collectively by members. As did so many others, he started with greater efficacy of the product and built from there: “First, being cured or maintaining health, second could be improved life and longevity and overall patient well-being, third could be an overall reduction in health costs to industry or the payer – for example, less money spent on other medicines, fewer visits to the doctor, fewer and shorter hospital stays – and finally, can the patient go back to work, and are carer costs considered?”

Members suggested that each source of value was aligned with a particular budget: the pharmaceutical budget, the health system budget or other governmental budgets such as social security or pensions. One member underscored the alignment when he said: “If you talk about value for money, it’s always the question of what money you are talking about. Are we talking
about money in the pharmaceutical sector, in the healthcare sector, or are we talking about money in society as a whole?”

Finally, members implicitly compared perspectives on value in their own Member States to those in other Member States. One member emphasised: “It is important to design a value assessment to the targets of your own system. Value for money in our system will be understood differently from other systems”. Another added: “You can’t separate value from the way the system is organised – financing and structure of the system affect interpretations of value”. Members most often spoke about the differences between the French and the English systems. They were mentioned as being the most explicit exemplars of focus on therapeutic value and health system value, respectively. Sweden emerged as the most representative of taking a broader economic and societal approach to valuing new drugs. Appendix 1 includes further elaboration of the spectrum of value offered in advance of the meeting.

**Societal value presents a conundrum**

During the meeting, members were asked to write down possible measures of each source of value. A compilation of responses is included in Appendix 2. Although members shared measures across each source fairly equally, they directed their energy foremost at debating societal value and the ability to measure and capture it. Members agreed that “social value is very important” and one member summed up the sentiment of the membership by declaring: “You couldn’t say the contrary”. At the same time, another member noted that “societal value is very complicated”, “not at all straightforward”, and “a double-headed weapon”. This characterisation summed up the tension between the significance of societal value and the necessity to consider it in value assessments on the one hand and its inherent difficulty to measure and capture on the other.

The tension became readily apparent as members debated three key elements of societal value: unemployment, carer considerations, and interestingly, innovation. Throughout the debate, members remained cognizant of how “very precious” the “social solidarity of European healthcare systems” is. They considered implications for the elderly and the sickly in their discussion of unemployment and carer considerations. Also implicit in the conversation were varying perspectives on whether to consider these elements from a macro perspective or an individual perspective.

Members were divided as to whether unemployment should be considered in assessments of value. One member took a macro perspective when he asserted: “The more people who are economically active, the more jobs you create in the system. Economically inactive persons are a drain on the system - you have to look very strongly at the ability to keep people economically active”. Another member countered that “there is no need to take a macro view [of unemployment]”. He argued: “If you have a technical person out for two weeks, it’s not easy to replace this guy. The practical benefits are clear – there is a very immediate, real impact”. Yet another stated succinctly: “If a drug makes it possible to work – that’s a good thing”.

A different set of members disagreed with assessing the value of a medicine in terms of its impact on unemployment. One member said emphatically: “I think we should absolutely avoid looking at societal value from the unemployment point of view”. He was among those who questioned
the ability to measure and capture the economic benefit of employment. He cited the elderly, who by definition are not employed, to caution against violating social solidarity principles and to highlight the challenges of measurement. He believed that considering unemployment would implicitly mean “that you are valuing the life of an elderly person differently than a working person” and “if you analyse savings from the use of a drug in terms of impact on unemployment, you can’t make any calculations”. He concluded: “I don’t think that lost work days is the right way to look at this at all”. Another member acknowledged that “drugs allow people to work” but argued that “unless you have full employment you are just shifting deckchairs around on the boat”. This supported an argument made by some members prior to the meeting that if an ill person is forced out of his job “someone else who is unemployed will move into that job”.

The debate regarding unemployment, while lively, went largely unresolved as did the ensuing debate about carer considerations. In fact, the discussions of each were intertwined and surfaced similar issues. Some members argued that little was lost economically because carers were not active in the workforce: “There’s the whole question about reduced carer time, but the majority of carers are already retired”. Members also questioned “how to work out the accounting of it all”, and one member spoke for himself when he acknowledged that “the economic advantages are greatly in favour of an early death. As I get nearer to retirement, I am very aware of this - no one will have to pay out my pension”.

On the other side was a strong belief on some members’ parts that carer considerations receive short shrift in the evaluation of new medicines. While they pointed to the economic consequences, they couched their comments primarily in welfare-related terms. Members said: “We know there is an enormous cost to both the health and welfare systems if elderly persons are sick for a long time” and “Everyone knows if you can shorten the period of disability in an elderly person’s life, you should do that”. Referring to the potential economic savings associated with reducing carer time, one conceded that “perhaps you don’t save money there, but it’s a question of welfare. Even if you have a pension, it’s a hard life to care for a relative. This becomes a question of quality of life”. Others cautioned against “crystallising social value into accounting of healthcare costs”. As one said, “It’s dangerous to get into a situation where the value of a person’s life (who has contributed for his entire life) is determined by his state at the end of his life”. After all, “There is a social contract – the fact that it is Alzheimer’s in your 80’s or asthma in your 30’s shouldn’t matter”.

Finally, one of the more interesting elements that emerged from members’ writing down measures of each source of value and in the ensuing conversation was the importance of an innovative industry as a societal benefit that should be taken into consideration when assessing the value of a medicine. This is consistent with one of the overarching objectives of the Network to reinvigorate industry competitiveness in Europe as well as one of the goals of sound drug policy to promote innovation. Members have affirmed the “need to put the conditions in place to have a strong industry” in Europe, and indeed a couple of members indicated that their “unashamed objective” was to “get the most value for the industry in [their own countries] and in Europe”. One member believed, “I think societal value can be measured and taken into account”. He recognised the multiplier effects of – and virtuous cycle created by – investing in research and development and argued that the industry “generates a lot of jobs, pays a lot of taxes and depending on where production and R&D are located, the value of the drug can be
measured differently”. Another government member in reflecting on the alignment between value and price of drugs suggested that “we don’t take account of innovation enough” and implored fellow members to think more broadly in this regard.

Measuring and capturing value becomes increasingly difficult with attempts to incorporate the wider spectrum of societal value

Because the implications are so vast and the pressures so great, several members argued that focus should remain on therapeutic and health system value. Two advocates of this position stated: “I propose to stay away from the right hand side [of the spectrum in Figure 2]” and “We shouldn’t take a broad view. We should limit ourselves to indicators that are purely health-related (ie, direct costs and direct benefits)”. Others echoed these sentiments, arguing that therapeutic value and health system value were more readily measured than societal value. One member offered the measures he deemed appropriate for each category of value: “The measure you can give to therapeutic value is simply the clinical effects – either from the ex-ante or ex-post studies. In measuring therapeutic value, you cannot look at money. However, money is the only acceptable way to measure the overall benefit of a drug and its value to the health system. I do think societal value of some drugs does exist – you can say in general terms that when people are cared for, they stay in work longer, are happier and their quality of life is better – but I do not think we can equate this value with the value of the drug. Thus, we couldn’t use [societal value] for decision making – which is the only purpose of determining the value of a drug – to make decisions”. Another member concurred, alluding to the increasing complexity associated with demonstrating value as one migrated to the right of Figure 2: “We are able to fix a value in a therapeutic sense, but societal value is so wide and difficult to fix. We need to avoid the right side and be much more concentrated on fixing value on the two left sides”.

The desire to “avoid the right side” of the spectrum associated with societal value seemed most linked to the problem of silo budgeting that exists in national budgets. Members acknowledged that it is often difficult to account for value generated by a new medicine within the health system and stressed that it is particularly problematic beyond the health system. One member summed up the dilemma of trying to measure and capture the benefits that might accrue to different sectors of society or national budgets (ie, pensions, the social security system) or those that accrue to individuals, but not necessarily to society as a whole: “When a drug creates cost savings in different areas, the costs always turn up in the health care system. We don’t have the financial systems to take care of that yet, but I would very much like to see [such a system]”. Another member added, “I think it would be a revolutionary thing if we could better analyse expenditure and allocate costs [to match savings]”, and several yearned for systems that fostered “better cooperation between welfare and healthcare”.

This aspiration to achieve a more comprehensive view of assessing and capturing value that transcended health system budgets was consistent with a citizen-centred perspective that was emerging within the membership. Members foresaw a natural transition to a patient-centred view of value taking place in Europe; as one member put it, “an inexorable tide of patient demand will transform the landscape”. Members not only reaffirmed this perspective but they also suggested that it might more aptly be framed as a citizen-centred view that encompasses broader aspects of societal value. As one member put it, “Citizens will direct us to the view that
"value\ is\ in\ the\ societal\ direction". Other members emphasised how critical it was to incorporate a societal perspective, consistent with the broader citizenry, into assessments of value. One contended: “If you go into society and ask people to describe what would be most valuable, they most often cite drugs that cure cancer, extend life, cure Alzheimer’s, delay dementia, or vaccines that prevent serious disease. Ironically, all those examples have relatively less exposure on health system value and relatively more on societal value. I think it’s quite hard to ignore the right hand side”. Furthermore, he added: “All the signals into the research centres are to look into treatments of diseases of the aged – all the things that we are finding hard to value and potentially not worth valuing”. Another highlighted the “huge value associated with eradicating or diminishing inequalities in health” and cited specifically the “huge advantage of immunisation against cervical cancer, a disease in which there is a terrible gradient of mortality (10 to 1) between the poor and the wealthy”.

Members’ observations and questions summed up the conundrum. One remarked that “so long as the health system is the sole provider of funding, there will never be enough money to create the society where I want to live, but there are resources enough in society to create a society where I want to live”. Another believed, “It’s perfectly possible to incorporate societal value in assessments” but wondered, “How wide do you want to take it? Can society afford to do it?” He summarised: “This is a societal debate, and we should enter into it with our eyes wide open. We must consider the implications and pressures put on the public system”.

**Demonstrating value in the real world**

“Not everything that counts can be counted and not everything that can be counted counts”.

- Albert Einstein

The “pressures put on the public system” will only begin to be alleviated if value can be demonstrated and captured in the real world. Determining the potential sources of value of a new medicine on which relevant parties can agree is but one step to aligning value delivered with value rewarded. At the inaugural meeting, a member asked a question at the heart of the debate about value for money in pharmaceuticals: “How do we better align the price of drugs with their value to patients and society?” Another spoke to the magnitude of resolving this question: “It will be a massive leap for Europe to all agree the sources of value and a quantum leap to agree the price of those value drivers”. He suggested that “fifty out of one hundred drugs offer no [added] value”, and urged his fellow members: “Let’s determine the ones that do and reward those”.

Determining which drugs do offer the most added value in the real world presents a variety of challenges that must be addressed. Members first discussed the extent to which prices were aligned with value of drugs. Then, they identified the dichotomy between regulatory and reimbursement requirements as an impediment to alignment. Finally, they discussed the data collection mechanisms required to begin to resolve the misalignment.

**Aligning price with value**

Electronic polling was used to calibrate the extent to which members believed prices of drugs were aligned with the value they delivered in the market. Members were asked to rate on a scale
of 1 to 5 (1 being ‘extremely well aligned’ and 5 being ‘extremely poorly aligned’) their assessment. No members selected the extreme positions; 47% and 33% believed that drug prices were ‘somewhat poorly aligned’ and ‘neither well aligned nor poorly aligned’ with value, respectively, while only 20% indicated that drug value and prices were ‘somewhat well aligned’.

Members suggested that the perceived misalignment between price and value emanated, in part, from three factors: the lack of shared definitions of value, the lack of transparency regarding both value and pricing, and the overemphasis on price. One member remarked that “if we don’t agree on the definition of value, how could we know if the value is aligned [with price]?” Another member attributed the misalignment to a lack of transparency: “I have no transparency on what the measures are for value…or for prices in my country”. Before the meeting, a member had summarised the problem: “In [my country] we have no experience in measuring value; we just keep looking for price, price, price”. Even a member who believed that price and value were ‘somewhat well aligned’ relied narrowly on therapeutic value in his assessment and suggested that broader societal factors were elusive: “If we define value as therapeutic efficacy, I think it is quite aligned. However, I can’t say we are extremely well aligned because we don’t take account of innovation enough”.

Many members agreed with the words of one member who said: “Objectively speaking, there’s no relation between the value of drugs and the price that is paid for them”. One member elaborated on the difficulty of “taking account of innovation” and otherwise aligning value with price. He said, “What you pay for on the drugs market is not value but value added” and added, “There is no monetary, arithmetic relationship of added value in the dollars and cents or euros you remunerate innovation with. You are prepared to pay more, but you cannot calculate how much more”. Another member posited that in some cases, payers are prepared to pay disproportionately more than seems reasonable. He contrasted vaccinations and cancer drugs to argue that there is “almost an inverse correlation” between price and value of drugs: “Perhaps the most effective things we do are vaccinations and treatment of hypertension. Those are far more effective and very, very cheap [relative to cancer drugs, for example]. Yet we spend huge sums of money on treatments that prolong life for a month, six weeks, eight weeks. The difference is absolutely startling”.

Implicit in members’ reflections on the lack of correlation between value and price was the significance of negotiation in setting of price. Since price negotiations often lack sufficient information to determine a medicine’s ultimate value in the real world, it remains challenging to align value and price. If value could be mutually agreed and directly calculable at the time of the negotiation, it is likely that price would somewhat more transparently emerge. However, as one member observed: “Aligning pricing to value is a matter of some negotiation – sometimes direct and sometimes indirect. It’s the setting of a market price by industry and the setting of a reimbursement price by government”. Somewhat more distressing, according to this member is that these two prices are “interconnected but I don’t think it’s a negotiation about value; it’s a negotiation about budget”.

A fellow member acknowledged the importance of the budget and the role it played in negotiation: “We argue about price, but the reality is payers are concerned about cost and industry is concerned about revenue”. He acknowledged: “We spend enormous amounts of
time on price, but price sends signals which torture all of us – price on its own cannot fix everything in the system”. He also began to allude to the importance for industry of achieving access to broader, more appropriate patient populations as well as payers’ emphasis on ensuring that drugs get used appropriately: “Price isn’t the whole discussion. We have to take the volume dimension into consideration”. Another member supported the view that with the emphasis on price, “too often each side has its [desired] price, but does not have its [required] patients”.

**Regulatory requirements versus reimbursement requirements**

Prior to the meeting, members and subject matter experts sought explanations for the misalignment between value and prices of new medicines. They surfaced the stark dichotomy between the requirements set by regulatory bodies to demonstrate safety and efficacy in order to acquire a license and the requirements set by reimbursement agencies tasked with getting the best ‘value for money’. One member emphasised: “Regulatory requirements are very different from reimbursement and pricing”. Another clarified: “Regulatory is efficacy, quality and safety [whereas] reimbursement looks at incremental value (ie, is there more value than currently available?)”. A member pointed out the differences in expertise relied upon in the two different types of decisions: “In our country, regulatory decisions are based on scientific evidence from specialists and academics. When you get to reimbursement, more economics comes in”. He asked, “Can you add other disciplines? Can you speed up the process?” and recommended “it would be nice to have an experiment” whereby people from various disciplines were involved to increase “leverage between the two parts of the pipeline”.

Members also suggested that pharmaceutical companies were hampered in their efforts to demonstrate value to reimbursement authorities, in part, because they expend tremendous resources “concentrating on benefits as determined by regulatory authorities”. A member lamented that too often, “industry brings in an innovative drug, [requests] a price and yet doesn’t have the data to prove the benefit”. Another added: “For many years, [industry] could access the market without making a strong, transparent link to value”. Members conceded that a focus on clinical trials resulted in “people making value judgements based on data [from clinical trials] that are completely ill-suited for the purposes [of assessing added value]”. After all, as one member put it, “in clinical trials, you often don’t have any real outcomes – you don’t get endpoints that have real value”.

Members argued the inherent limitations in clinical trials were exacerbated by the dominance of the United States’ marketplace in influencing how resources for studies are allocated. Members recognised that “the influence of the FDA is a complicating factor”. One member said: “Because the FDA is so prominent and requires only placebo studies, resources get spent in the United States”. Members and subject matter experts believed that the prominence of the FDA was one of the impediments to more widespread use of head-to-head studies across Europe. Increasing numbers of Member States request them in their reimbursement processes, and many members agreed: “The idea of more head-to-head studies is good and should be realised” and “We need [them]”, but “there has to be a clear requirement for them”.

Based on the conversation at the meeting, it appears as though industry has heard the message. Industry members said that although “comparator [trials] are very difficult”, “when we compare new drugs, we use comparators and we make head-to-head comparisons”. In fact, one member
asserted: “Right now, we only do comparators”. However, simply agreeing to comparator studies is not the entire solution. Debates about choice of comparators and the general usefulness of clinical trials in reimbursement decisions persist. One member pointed out: “The problem is the choice of comparator. It’s not easy choosing the standard”. Members acknowledged: “Different countries make different choices about the ‘most meaningful’ comparators” and “Some choose the most frequently used drug in a class, others choose the most recently introduced and still others choose the cheapest”. One industry member summarised the challenge: “If we need to fulfil everyone’s requirements from the start, we have burnt out patent life”.

Finally, a key difference between regulatory and reimbursement requirements is that “the timing is different”. Members emphasised that often the total value of a drug can only be demonstrated through its use, over time in the real world. Members commented: “Clinical trials are short-term and often the benefits [ie, value of a drug] come long-term” and “You have really got to look at value on an iterative basis over time”. Others added that it takes time to resolve the uncertainty of how a drug will perform in real life settings as opposed to in the controlled environments of clinical trials. They observed: “Clinical trials are essential to isolating the impact of what the medicine might do, but do not answer the question of how it will impact patients under the conditions of the real world” and “Until you have applied a drug in a broad range of patients and had a statistically significantly sample you don’t really know the value [and to whom]/”. Several members advocated efforts to enhance alignment between regulatory and reimbursement authorities in order to bridge the gap between the two. A member identified the fundamental objective of such efforts: “The aim is to get drugs to market quickly to a broader cohort without loss of safety and efficacy. That would enable us to understand value across the full spectrum”.

Data collection requirements

Members recognised that better collection, access to, and analysis of data were required to determine the longer-term, real-world benefits of new medicines and to fully “understand value across the full spectrum”. Despite the sharp focus during this meeting to “work collectively to define value”, members acknowledged that shared definitions and methodologies for assessing value have limited utility in the absence of shared data. All are required to effectively demonstrate and capture value and align value with price. Members agreed: “We need to collect better data over a longer period of time” and “National databases will be vital because you can capture data on safety and efficacy and do so against a pricing mechanism”. They highlighted the need to work collaboratively across countries and stakeholder groups to develop trusted, shared data collection systems and to facilitate effective post-launch evaluation of medicines.

There was consensus that the first step in establishing shared data collection systems in which “countries can accept data coming from other countries” is to “define what people want”— to “agree on the kind and quality of information we want and use some of the same data and guidelines”. Before the meeting, an industry participant highlighted what was required: “We need to work in a collaborative environment. We should agree on protocols regarding what we are going to measure, for which patient sets, what constitutes successful outcomes, and so on. It would be good if we could agree with payers on what we should be monitoring”.

Value for money in pharmaceuticals
The next step was to “get to a point of producing relevant information for the end user of that information” and to agree that “the source of that information is credible”. However, agreeing upon a trusted source of the data is difficult. One member asked “who trusts who to come up with the information we need to go forward?” and was “really struck” that key decision makers tended to “discount as a credible source of information what comes from industry”. He further noted that “on one hand, that’s completely understandable, but on the other, that’s the most knowledgeable source of information”. Members also questioned the extent to which data could be shared and accepted among payers from different countries. One member responded: “When it comes to therapeutic value, yes – that is the same everywhere. For cost-effectiveness, maybe – perhaps we could model it by inserting county-specific data. It’s a question of time and money. It may be worth considering”.

Upon hearing this, a member suggested that “we are now touching on the real problem of drug launch in a European market”. While acknowledging that “you will obviously never have a common system of acceptance – of the same price paid in the same way across the board”, he stated: “It would be interesting to have a certain number of shared concepts and shared methods of assessment. There are savings to be achieved if joint studies were done in one country and recognised by other countries. If we had a hub of countries that would agree [on a shared methodology for data collection], it would be easier”. He concluded: “Each country would still be free to make its own pricing decisions, but at least we would have a large folder of information [that was shared]”.

Joint studies and data collection would facilitate post-launch evaluation of a new drug. Members acknowledged at the last meeting that a focus on health outcomes for patients would require assessments of the effects of medicines in practice within healthcare systems over longer periods of time. A member indicated that in his country, they were “looking at mechanisms to make it possible in an effective way, to draw consequences from real-life, post-launch studies”, but also cautioned “you have to be aware that not everything is possible”. Payers acknowledged that industry is in a bind over the requirements for specific data: “We are aware that harvesting data is very costly for industry and for government” and “It’s very difficult for us to ask industry to give us all their data. But if more people asked for the same data, we are more likely to get that data. You can’t make everything uniform, but you can broadly say some drugs have some benefit across all our populations”.

Finally, members agreed that integrated information technology systems that incorporate shared concepts, consistent methodologies and trusted data for assessing value would benefit all stakeholders by enabling clear demonstration and capture of value of a drug in the real world. One member outlined his vision for such a system: “During the conditional approval phase, what could happen is you could use the database to select appropriate patients for the drug. On GPs’ screens, if they call up the drug list, they could see every patient in their practice who is suitable to receive the drug. This would be a huge benefit for industry. The Vioxx problem wouldn’t happen - industry could target so that only the patients who would benefit, would get the drug. Useful drugs could stay on the market. Also, these data would be useful in identifying additional appropriate indications for drugs”. Another member concluded: “Implementation of this [vision] could lead to value-based versus wish-based prices”.

Value for money in pharmaceuticals
Risk sharing arrangements as an example of value-based pricing

“To win, you have to risk loss”.

- Jean-Claude Killy

One mechanism for achieving “value-based rather than wish-based pricing” is risk sharing. Members reiterated that linking price to value is difficult in pricing and reimbursement systems because the full value of a medicine is not always known at the time the price is agreed. Risk sharing arrangements allow a drug to be put on the market at a “reasonable” price with the agreement that additional data will be gathered over time to demonstrate the value of the drug. Depending upon whether or not the drug yields its anticipated outcomes, its price could go up or down or other dimensions like volume could be adjusted.

Members generally supported the concept of risk sharing because it relies on real-world evaluation to strengthen the connection between value and price of a drug. In support of real-world evaluation, a member said: “I firmly believe that if there was more emphasis on real-world evaluation, we would have to be more clear in articulating where the value is derived”.

Government and industry members shared their support for risk sharing, respectively: “It would be better than the existing system - it would be possible that after the drug is in the market to come together with industry and to pay for existing value. It would be a more flexible way of price finding and setting in the market” and “Risk sharing offers a chance to demonstrate the value of a drug and to demonstrate that its price is justified”.

Although members agreed in principle that risk sharing was a good approach in certain circumstances, they also agreed that it is not a model that applies to all situations and that these arrangements must be considered thoughtfully. They said: “It’s not a solution to every drug – there are not enough patients, money or time”, and “The devil is in the details”. There was both curiosity and scepticism among members. These were exemplified by one who requested: “I would like to see some concrete examples of what we are talking about. What sorts of drugs? What sorts of conditions? What sorts of data? Agreeing in principle is dangerous”.

During the meeting, members shared examples of risk sharing from their own experience as they addressed some of these important questions. First, they discussed why risk sharing should be pursued and identified corresponding risks for each set of stakeholders. Next, they explored how contracting would work to maximise potential benefits, mitigate risks and change the nature of reimbursement negotiations. Finally, members amplified the need to foster collaboration to ensure alignment of stakeholder interests and to capture value from such arrangements.

Why pursue risk sharing arrangements?

Risk sharing arrangements are intended to more effectively align the shared interest of payers and industry to deliver improved health outcomes to patients in the real world. However, the underlying considerations are complex, implementation challenging and the rewards uncertain. There are risks for all involved: payers, industry and patients. The big challenge is to determine the benefits and circumstances in which these agreements should be pursued. When asked why risk sharing agreements should be pursued, members pointed to the perspectives of the three stakeholder groups highlighted throughout the day’s conversation.
One member alluded to the vantage points and associated objectives of each set of stakeholders when he speculated that there were four reasons that governments would engage in these agreements. The first reason related to patients, the second and third to payers and the fourth to industry: “Governments do this sort of thing for a number of reasons. First, it gets them off a painful hook. Patients might be screaming for it. That’s at the crudest level. Second, there may be some interest in finding out if the drug is what it claims to be. Next, they try to satisfy themselves that there is value for money. Fourth, it allows governments to demonstrate that they are still supporting industry and innovation. I think it’s a combination of all four, but mainly the first and fourth I think”.

Other members reflected on this reasoning and tried to put themselves in others’ positions by highlighting the benefits to industry and to patients. A government member conceded that “maybe there’s a little bit of postponing the ordeal” related to “screaming patients” but stressed “there is another aspect I think”. He continued: “From an industry perspective, it is able to access the system more quickly and achieve payback sooner”. Such arrangements enable faster access to the market for drugs that might otherwise be held up in protracted negotiations.

Members agreed that there is a powerful incentive on the part of industry to preserve patent life and that ‘access to market is critical’. The long-term value of a drug to industry in terms of the return it will yield on its investment is predicated on its speed and breadth of uptake. An industry member summarised the dilemma: “The life expectancy of a product is shorter than it used to be. There is huge pressure to access the market as quickly as possible. Risk sharing allows earlier access without having to go through another round [of negotiations]”.

Members noted that risk sharing served to reconcile differing beliefs between industry and payers about the alignment of value with price of a drug. Industry members believed these arrangements were helpful “when you have mismatches in expectations [between industry and payer], where you don’t have the data yet”. With risk sharing arrangements “you have a chance to prove [the drug’s] worth and at a European price you want”. A government member added: “The question occurs when we are not sure whether it is innovation or not – [if we were] definitively certain, we would not go to such lengths”. One industry member offered his thinking: “First, if you are confident from controlled studies that you have real value, but government might not see it – perhaps no good surrogate markers exist. Second, is if you have something that totally transforms the standard of care, but the payer doesn’t see it because it doesn’t come out of his budget per se. Our response is ‘let’s measure it over time and prove the reductions in cost’. If we demonstrate success, it’s a big plus for us – but there are benefits for both”.

One member was explicit in reminding his fellow members that these arrangements didn’t involve only payers and industry. He said: “Let’s not forget the patient. There are three parties here – patients may see medical benefits as well”. Several agreed and supported the idea that risk sharing agreements accelerate access to medicines, especially for those in greatest need. The studies demanded by risk sharing arrangements also help all stakeholders acquire knowledge about diseases that they might not otherwise gather. One member noted that even if the study is conducted only in one country, “there is benefit for that country and the data we generate will be a benefit for patients across Europe”. A member referred to payers, industry and patients when he stated simply: “Overall, it’s a win-win-win”.

Value for money in pharmaceuticals
A member amplified the perspective of each stakeholder and touched on the spectrum of value as he related an example in which he was personally involved. In this case, industry had requested a relatively high price on the basis of benefits to patients and savings to the health system resulting from enhanced compliance of a new medicine. The government member recounted his experience: “Industry came claiming that they should get a price four times higher than normal. Our initial reaction was ‘we’re not interested, it’s too expensive’. That was our first reaction, but we had second thoughts because of the extreme seriousness of the disease in question. If it was true that compliance could be improved and that could result in a drop in relapses, you have benefit for patients and also an economic gain”. He understood that “if [the claims about compliance are] all true, this is something that cannot be demonstrated in clinical trials” because if patients were not compliant, they were dismissed from studies. He recognised “you need something else”, and in this case, “something else” was a risk sharing arrangement in which industry would conduct a rigorous, real-world study designed by the payer to examine the effects of use of its drug on compliance, relapse and hospitalisation.

All stakeholders share the risks

As its name implies, there are risks for all involved in these types of arrangements. An industry member acknowledged that “industry bears some risk as does the government” and another member added, “It’s not risk-free for patients either”. Although members touched on several risks, those that were most prominent in the discussion were the financial risk borne by government and industry and the risk faced by all stakeholders that the drug might have to be withdrawn from the market at the end of the study period because it did not demonstrate sufficient value. Finally, members discussed the challenges industry might face if it was requested to conduct similar studies across Member States.

Both industry and governments bear financial risk related to price, but there is greater uncertainty related to the possibility of having to withdraw the product from the market. A government member acknowledged that industry “at least in the transitional period, is not making as much as the price it originally asked for”. A member added, “There is financial risk for the payer too” but perhaps more significant is “the risk of publicity”. One government member shared his concern: “We have to publicise the price and we are held accountable for it. How am I going to demonstrate that the drug should stay on the market and that it should be at that price?” One member shared a personal experience to make the point: “When a product in a large observational study didn’t yield the expected results, we found ourselves in a very embarrassing situation. The drug wasn’t effective, but there was no question of withdrawing it from the market – the problem was the product had been priced and there were already patients on it”.

One member indicated that it was possible in his country to withdraw a product that has not demonstrated value: “It is possible to withdraw reimbursement – it is part of the [national] system. We sometimes limit reimbursement in time and demand more data. We can withdraw [the product] if the new data shows negative outcomes”. He added that “this is the negative side – that we might have to withdraw – on the positive side, we can we can give faster access to new drugs than if we didn’t have this possibility”. He further emphasised: “And the company can apply again if they have more data showing the drug is better than they had [shown] at the start. Then we can allow a higher price”. This system gives one the flexibility to err on the side of
accepting a drug for reimbursement earlier than one might otherwise and facilitates the kind of thinking a member expressed at the inaugural meeting that “mistakes might be made either way initially” but “it is easier to live with making a mistake by reimbursing something that turns out not to add value than not to reimburse something that turns out to be worthwhile after all”.

Finally, a very real risk for industry is that the burden of conducting post-launch studies might become more onerous than it is worth. While one member suggested that “if industry is successful in demonstrating value, this will be noted by reimbursement boards across Europe”, another countered that this might lead to “a tremendous logistic challenge that becomes a dilemma”. A government member pinpointed the challenge when he asked his industry colleagues: “I wonder if it is done in one country, wouldn’t a risk be that all 27 countries would want it? How would you handle that?” An industry member conceded: “It points a very strong search light on the challenge of doing this with 27 Member States – all potentially asking for a different test”. He and others highlighted how integrated data collection systems would offer “ways through that”. They said: “You may have several countries with questions, but a common method to make a demonstration” and “The [dilemma] is relieved enormously as you move from prospective clinical trials that are enormously expensive to observational environments with IT systems that exist to support them”. Unquestionably, integrated data collection systems across Europe will palliate this risk, but several believed this risk was overstated. A government member spoke for many when he said: “I don’t see [risk sharing] as a model that applies to everything. This is a model that stands completely outside the standard, outside the norm”.

### Benefits of risk sharing
- Accelerates market access
- Emphasises value rather than price in reimbursement negotiations
- Demonstrates health outcomes for patients in the real world
- Aligns value delivered with price and volume
- Inspires collaboration among stakeholders

### Risks associated with risk sharing
- Possibility of product withdrawal if it fails to demonstrate value in the real world
- Uncertainty of budget impact due to possible price and volume variations
- Additional costs associated with outcomes studies may outweigh benefits
- Other countries may request similar studies or delay access until outcome study results are available
- Healthcare delivery system behaviour and/or incentive structure is inconsistent with outcome study requirements
Effective contracting aligns objectives and changes the nature of the negotiation

A thoughtfully constructed contract aligns the potential rewards and risks so that it is a sensible arrangement for all stakeholders. One member spoke on behalf of several when he questioned whether or not it was possible to construct a contract up front that 1) allows adjustments in the price of a drug based on outcomes demonstrated, 2) accounts for stakeholder interests, and 3) obliges parties to a certain set of behaviours. He asked: “Do you think it is possible to have a system with a penalty and a bonus? Is it possible to set out the decision rules beforehand to make decisions later? You don’t want to have to negotiate about it later”. Several believed that such agreements were not only possible but essential to effective risk sharing. One said: “Absolutely, you can develop a contract that works, one that prioritises value to be delivered and obliges each side [ie, industry and government] to certain things”. Another added: “You need to agree on a contract up front, the parameters you are looking to evaluate and those that will mitigate risk [for both sides]. As best you can agree and establish at the outset, the better it will be in the end”. In addition, members suggested that this emphasis on identifying outcomes sought from a drug would shift the focus of reimbursement negotiations from price to value and might potentially lead to more discriminatory pricing.

Members focused in the discussion on one particularly challenging aspect of creating a contract: agreeing the study designed to evaluate outcomes. One member thought an effective contractual relationship was possible “provided there is an agreement between the payer and the producer that the test is a fair test”. He questioned: “How do you negotiate what is a fair test? What would be the criteria for success or failure?” The government member who had shared his personal experience earlier agreed that determining “criteria for success or failure” was the most difficult part of agreeing a contract. He referenced two key components of such a contract, the financial obligations and “the test”, and indicated that the most challenging part of the arrangement, surprisingly, was not the financial aspect but the negotiation over “the test”. He shared: “What led to the joint definition of the study, to look at the parameters we were going to choose, that was very hard. Then there was deciding the financial risk, and that wasn’t difficult. That wasn’t difficult at all”.

His experience suggested that it was possible to shift the focus of the negotiation from an emphasis on finances (ie, price, budget) to an emphasis on value created (ie, outcomes for patients). One member felt strongly: “Frankly, that’s where the negotiation should begin.” Harkening back to an earlier comment that “fifty out of one hundred drugs offer no [added] value”, a member posited: “Identifying a relatively tough set of hurdles is not unreasonable – there will be drugs out there that do not accrue incremental value”. Members understood: “A company makes the choice to engage or not. If we can’t agree the test, then the sensible company says I’ll take my chances in the Wild West’. If you can agree, then you go into the test and live with the consequences of the test”.

In risk sharing, the consequences of the test “create new dimensions for negotiation” by allowing for “a penalty or a bonus”. One member advocated “a discussion in which if your drug fails to live up to the promise, you pay a penalty, and equally, it would be reasonable that if the drug turned out to be substantially more effective than initially thought, there could be a discussion around where there [might be] broader population use or even a higher price”. The member
recalled the comments made earlier that “payers are concerned about cost and industry…about revenue” when he suggested that “this [discussion] offers flexibility - neither you nor we are really concerned about price”. He elaborated: “The negotiation of what flexes could be population size which is a bit below the radar screen anyway in terms of political or high profile challenges – it doesn’t have to be just price. [Population size] would clearly be manageable”.

In the minds of some members, “trying to open up new dimensions of negotiation” might lead to the possibility of more discriminatory approaches to pricing across Europe. Consistent with their fellow member’s earlier observation that negotiations are more about budget than value, several members lamented the state of the current environment. An industry member shared that “one of the great dilemmas for the industry in Europe across all Member States is the regular non-discriminatory price decreases regardless of the value of the drugs to try to allow budgets to be met”. He suggested that if the data showed that particular drugs weren’t offering added value that it would be preferable to have a discussion about those rather than to penalise all drugs with across-the-board price cuts: “It is better to evolve to a system that is more discriminatory as long as the discrimination is based on a reasonable premise of evidence and data. Compared to what we do today, I think that would be better”. Another member agreed it was unfortunate that “we don’t live in a lovely, ideal world where rational decisions and discrimination will win – we live in a world where we are constantly looking for the best compromises”.

**Collaboration among stakeholders is a key criterion for success in risk sharing**

Risk sharing facilitates a shift in focus from budget to value – in the form of improved outcomes to patients that ramify throughout the spectrum of value. Members recognised that this shift would require “developing stakeholder collaboration at the point of delivery”. It is implicit in a succinct description of “a contract that works – one that prioritises value to be delivered and obliges each side to certain things”. Prior to the meeting, members emphasised the need for integration of multiple constituents in the value chain to ensure the success of risk sharing. A government member summarised: “If the pharmaceutical industry would like to organise risk shares, then they will have to organise with the doctors and hospitals, as well. There is an integrated approach to delivery. You can’t just give the product and that’s it. I think we need to think of collaboration between doctors, hospitals, industry and [payers]”. Industry members concurred: “We cannot design and implement this on our own” and “We need assurances from government to sign up for the system risk associated with the contract”.

These comments shed light on a significant risk that has not yet been addressed: health system risk. A member summarised challenges for industry in attempting to demonstrate the value of their drugs in real-world settings: “Real-life outcomes are just as much linked to the health system and issues of access as they are to the efficacy of the medicine. Real-life data that show the usefulness of the drug are closely linked to the larger healthcare system and to behavioural issues”. During the meeting, members shared examples from their Member States of constructive collaboration among stakeholders designed specifically to enhance outcomes for patients. This discussion elicited principles germane to ensuring successful risk sharing agreements. Specifically, members focused on using medicines appropriately and creating incentives to drive behaviour change in patients and physicians.
Although much of the emphasis during this meeting was on aligning value and price of medicines, risk sharing highlights the importance of proper use of drugs within the health system. Members commented earlier on the need "to use drugs appropriately", and a member touched on this theme at the last meeting when he said: “A big challenge [of achieving value for money in pharmaceuticals] is to assess the connection between value, use, and price”. To amplify the connection between “value, use and price”, members stressed the importance of “creating a culture of appropriate behaviour” among patients and physicians. They said: “There is a behavioural problem at play here” and “We have to look carefully at how to induce changes of behaviour that change the cost base across the spectrum [of value]”. On describing initiatives in their Member States, members noted that the critical topics were “trying to better understand what drives the use of new drugs by physicians” and “involving physicians to influence prescription behaviour and patients to influence consumption behaviour”. Otherwise, “you have huge problems with capturing value”.

**Forward moving agenda**

“There is nothing wrong with change, if it is in the right direction”.  
- Winston Churchill

The second meeting concluded with members discussing and prioritising issues for future collaboration. They agreed to collaborate to advance the following two work streams over the months ahead:

**Develop shared value frameworks.** In this work stream, members will build upon the work they have begun in developing the spectrum of value illustrated in Appendix 2. They will define sources of value and develop methods to assess value across the spectrum. Members agreed that the work would be challenging, but worth pursuing: “It’s tough to make these value assessments, but that shouldn’t stop us from trying” and “It’s central to everything we are doing”. Another expressed confidence in the collective ability to create shared definitions and methodologies of value assessment: “I don’t think that after all there are such diverging views of therapeutic, health system and societal value. I think we can get there. I am sure we can get there”.

Members reinforced the desire “to get clear specifics of what constitutes value from multiple perspectives” and agreed to consider shared definitions from the vantage points of the key stakeholders (eg, industry, patient, and patient). Members also highlighted the possibility of considering additional key stakeholders in order to create a more holistic perspective of value. Members agreed “it would be interesting to know how physicians could play a role” and suggested considering the role of “investors and the public – who are different and have different values from patient groups”. They also agreed “to think fairly specifically”; they will test the utility of the definitions and methodologies by applying them across products and disease areas. The goal is not only to come to “mutual understanding” of value but also “mutual recognition” of methodologies of assessment. A member summarised the practical goal of this work stream: “We need to address [these] issues in order to improve the way we care for patients and manage diseases”.

**Develop guidelines and conditions for pilots and experiments.** Members are committed, in the second work stream, to developing guidelines and conditions for pilot projects designed to
assess, enhance and capture the therapeutic, health system and societal value from medicines. Pilot projects might include retrospective value assessments of specific medicines. Members thought that it would be valuable “to take one or two drugs that were introduced recently and see what would have happened” had proposed value-based pricing arrangements been applied to them. Pilots might also include establishing new models of public-private collaboration to capture broader health system and societal value. Just as with developing value frameworks, members were similarly eager to develop guidelines and conditions for pilots that would incorporate the perspectives of various stakeholder groups as well as address specific products and disease areas. Members captured this sentiment when they stated: “We have to make the conditions clear before we start what the roles will be for all parties – government, patients, industry – and identify under what conditions they would want more pilots” and “Let’s target a couple of important disease areas and products”. Members believed these pilots will accelerate learning and lead to a more innovative healthcare environment across Europe.

Conclusion

“The important thing is this: To be able at any moment to sacrifice what we are for what we could become”.

- Charles Dubois

We close this ViewPoints by reiterating that members are not satisfied with the status quo. They recognise that change is essential and have committed themselves to sacrifice the certainty of the status quo for the uncertainty of a potentially more constructive and collaborative future. Members achieved their meeting objectives to advance collective understanding of value for money in pharmaceuticals and to consider existing examples of risk sharing agreements designed to better align value with price of new medicines. While there is much left to be done, they demonstrated an “openness and willingness to work toward mutual understanding of value and to start experiments”, and they are committed to continuing their collective effort. Members believed: “I think we made good progress and we’re going in the right direction” and “I do think we are swimming with the tide, and it is worth pushing on”. In addition, they are committed to pursuing change that will have “practical impact” today while bearing in mind that actions taken today have ramifications for the future. One member said, “I think the long-term strategic perspective should be kept in mind”, and another encouraged his fellow members to engage in a proactive conversation that “takes the perspective that looks back from 15 years hence and asks ‘what do we want to put in place today to have the kind of future we want?’”. It is this forward-looking perspective that will enable members to “reconstruct relationships” that will “allow industry and government to interact more constructively” on behalf of Europe’s citizens.

About this document

The views expressed in this document arose from discussions with the members of the European Healthcare Innovation Leadership Network, a group of leading stakeholders from the public and private sectors committed to improving healthcare and economic wellbeing in the European Union and its Member States. This document is not intended to represent the particular policies or positions of the Network’s individual participants or their affiliated organisations.

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Appendix 1- Excerpts from PreView related to the spectrum of value

Therapeutic value at the core

There was strong consensus that any assessment of value of a new drug begins with determining its therapeutic value against existing medicines. Members focused on “high unmet medical need” and the need to “assess therapeutic added value first”. They emphasised the need to determine “the clinical outcome to show that one drug is better than the other” and to “first and foremost, demonstrate that something is more efficacious than the ‘gold standard’”.

According to members, therapeutic value can take on many forms. As one member noted, “therapeutic outcomes can include increasing efficacy, increasing longevity, stemming disease, controlling symptoms, and reducing side effects”. Another elaborated: “It is a composite concept but at the end of the day, it is driven by hard data and clinical endpoints, namely efficacy differentials, safety differentials and dosage improvements”. And yet another stated: “I want to know ‘what is the extra [medical] value [the drug] will bring’”?

Although there was core agreement around the importance of patient outcomes, there was greater variance in the criteria members used in order to determine where positive patient outcomes can be judged to be realised. Definitions and categorisation were not necessarily shared. For example, while easier dosing was viewed as an enhancement to patient quality of life for some, to others it related only to compliance. Whether easier dosing was viewed as patient convenience or as contributing to compliance had bearing on how it gets valued. One member pointed out: “Reducing from four times per day to once a week translates into comfort for the patient, but that doesn’t mean the value of the drug doubles – we may pay 10% or 20% for that, but not double”. On the other hand, the same member pointed out that in the case of mental illness, for example: “It may be necessary to change to once per week because that increases compliance and that, in effect, translates into an increase in therapeutic value”. Thus, issues of patient ‘comfort or convenience’ were valued less than the very same attributes that bore on compliance.

Members and observers pointed to France as being the most explicitly focused on added therapeutic value in its assessments of value. Emphasis on broader health system savings takes on a less important role and societal benefits even less so. Highlighting the emphasis on medical added value, a French observer cited cancer drugs: “Sometimes we know when we pay for a drug that there is no other advantage than to delay death – this is a good result, but it is purely medical; we must not think that we are paying for savings elsewhere or for anything other than medical benefit”.

In contrasting the French with the UK system, one member asserted: “There are only two comprehensive systems – France’s and the UK’s. In France, it is about the best drug possible, regardless of the cost. In the UK, a drug can be too expensive, even if it brings about great health benefits”. He went on to allude to the QALY in his comparison: “In the UK, a year of life has a certain value, and this is translated into a price. In France, a year of life for a person with cancer is no less valuable than a year of life for a person with MS. It is black or white – either a new drug at a certain price holds value or it does not”.

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Value to the healthcare system

Industry works diligently to demonstrate to payers the broader set of benefits beyond pure therapeutic outcomes and effects on the pharmaceutical budget. Yet, even those benefits that might be gained by other facets of the health budget are often dismissed as “illusory” by payers and governments because of the difficulty in capturing them. This view limits payers’ interest in accepting new drugs for reimbursement because the broader economic value to the healthcare system is not something that they can immediately capture. Unfortunately, as one government member stated: “We are so eager to get value for money in pharmaceuticals, but in no other facets of healthcare”.

An industry participant elaborated: “For example, if you develop a drug that stimulates the body to make platelets, thereby reducing the risk of disease and potential adverse reactions associated with regular platelet transfusions, payers ask ‘why should I pay for that? I have plenty of people who are willing to donate platelets’. The infrastructure for platelet donation is already in place and operating smoothly and there are no incentives for payers to disrupt their systems”.

A member contrasted drugs with medical devices that more easily demonstrate the savings in hospital costs: “Devices are narrowly connected to surgery, so it is much easier to prove that they will afford savings in the hospital. To do this with drugs is very rare. It is not good for industry to push this [savings] point too heavily”. Another member was equally direct: “The question for me as a payer when I buy a new drug is not ‘will it save me money?’ That never happens”.

Other members expressed the difficulties they face when seeking to realise cost savings in the healthcare system. In the words of one member: “If there are proven economics about using a drug to prevent hospital costs, then [payers] will be very interested in seeing this. To be honest, I have not seen very good analyses like this”. Another government member added: “Big pharma will say that [its new drug] can diminish stays in hospital – we say, you may be right, but we will never shut down hospitals…”

While members acknowledged that shutting down hospitals or even wards can be challenging for health officials, they offered a couple of recent examples where “a drug can be very expensive but prevents a lot of costs in the hospital sector”. The same member who suggested that the pharmaceutical industry should not “push this [savings] point too heavily” acknowledged that “proton pump inhibitors [PPIs] and HIV drugs are a reality of this [i.e., drugs yielding significant health system savings]”, but he also noted that “they are exceptions”. To be sure, both cases required some measure of patience in yielding their full benefit. One member pointed out that in the case of PPIs “we didn’t know at the beginning that patients could keep taking these and ulcers would stay away”, while an industry participant highlighted: “HIV wards weren’t closed down instantly, but they were in the course of two or three years. You can’t [always] capture [savings] instantly but you can in longer time”. A government member summarised: “If you have governments that have to be convinced, it must be very good data I think”.

Whereas France is considered an advocate for therapeutic added value, the UK is considered an exemplar of systematic focus on the health services budget. When members and others spoke of the UK, they mentioned NICE (The National Institute for Health and Clinical Excellence), the independent organisation responsible for providing guidance on new medicines, and its reliance
on the QALY (quality-adjusted life year). In the words of one subject matter expert: “NICE looks at the health gains, the quality of life gains, and the other benefits a drug can offer. They do this through the QALY. They compare the benefits of a drug to its potential costs. It is an economic model, set in economic terms”. Another respondent explained: “In the UK, value for money has to do with the measure of efficacy of the medicine and the money spent on healthcare. The objective is to get maximum health benefits or outcomes from a limited and constrained budget. This gets distilled down to a pure economic measure called the QALY — which is a number between 0 and 1 where 1 is a state of perfect health”. Some expressed concerns over the use of the QALY, suggesting that it fails to capture broader societal concerns: “It is difficult to condense health outcomes to a numerical value. QALYs don’t take into account societal factors—the overall spectrum of impact. This is a shortcoming”.

Societal value

Taking into account a broader spectrum of value can be especially challenging. Nonetheless, a member emphasised the importance of taking a societal perspective in assessments of value: “One big gap in the system is that public authorities are buying healthcare and they tend to look purely at cost and don’t look beyond the immediate impact on the health budget whereas people who improve in their health can become less of a burden. They and their carers can contribute to the economy. You have to look at the broader benefit of spending more on healthcare for the economy”. Industry participants questioned “how governments pay attention to more social, humanistic endpoints” and expressed concern that “from a humanistic perspective in Europe, payers don’t pay that much attention to societal factors”. A member underscored the importance of “constructing a more holistic view of the value chain” and the “need to credit caregiver economics”, for example.

One member offered an example of the conditions under which the benefits of this broader societal perspective would be most apparent and noted that it would be well rewarded: “We are all hoping for the golden pill that we can give to elderly in homes to cure Alzheimer’s. We hope that one day the golden pill will be found, and we will pay for it, and we will pay for it big”!

Despite this hopeful example, accounting for societal factors is especially challenging for both industry and payers. Members stressed the difficulty in measuring benefits that may accrue in different sectors of society or national budgets (ie, pensions, the social security system) or accrue to individuals, but not necessarily to society as a whole. Members argued the ‘increased productivity’ argument loses its weight because “unless there is full employment, someone else who is unemployed will move into that job”. Similarly, there are limitations in accounting for carer time; one member asked: “In the case of Alzheimer’s, how do you account for it if the carer spouse is also retired?” He summed up the dilemma when he said: “Unfortunately, this is not the goose that is going to lay the golden egg”.

Among Member States, Sweden appears to be the most systematically progressive in attempting to capture this broader societal impact. Sweden bases its pharmaceutical decisions on three criteria: the human value principle, the need and solidarity principle and lastly, the cost-effectiveness principle. The Pharmaceutical Benefits Board, which decides whether or not medicines should be reimbursed, includes a description of the ‘societal perspective’ in its overview: “We also need to do more to get a full societal perspective. So we take into account if
the drug means that the patient can work and support himself or herself instead of being sick-listed and perhaps forced into early retirement. Here the benefits go to the individual, the employer and to the state which avoids the costs for sick-listing and early retirement. If the patient is older perhaps the treatment means that he or she can manage better without as much help from the municipality’s elderly care services or relatives”.3 It further emphasises: “The crucial aspect is instead that the drug is cost-effective, and not just for the healthcare sector, but for society as a whole”.

The patient perspective

While members believed that “value for money is based on patient outcomes, on patient reports” and that “the foremost principle is that the most important customer is the patient”, the aforementioned discussion of sources of value was grounded primarily in payer and industry perspectives. The tension between expanding access to medicines for patients and managing budgets was readily evident. As he is not the payer, the patient is unencumbered by this tension and thus, it is his perspective that unifies therapeutic, health and societal perspectives. In the words of one member, “patients have to be at the centre of healthcare, we must look at their needs and demands”.

An industry participant supported this view particularly as it related to medicines: “We are intimately interested in wanting to know how medicines we produce impact patients in the real world, ultimately to ensure that the right patients are getting the right medicines. This might seem altruistic of industry, but if the right patient is getting the right medicine, then we can sustain our business; if, on the other hand, the wrong patient is getting the medicine, then we will not be sustainable”.

Unfortunately, this focus on the patient rarely gives rise to systems in which patient preferences are fully taken into account. A member pointed out: “If the patient says one pill a day instead of three is an improvement, the payer may say differently”. Another added: “Payers are prepared to pay for efficacy, less willing to pay for safety and even less for a dosage benefit”. Nonetheless several members foresaw a natural transition in Europe toward a patient-centred view of value. One member summarised: “You have value for money in terms of the payer and value for money in the eyes of the patient, and I think that, ultimately, the second way will win. I think if we can have evidence that indicates more value for the patient, then no one will complain”.

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Appendix 2 – Members’ initial perspectives on measures of value

During the meeting, members were asked to share initial thoughts on measures that could be used to assess therapeutic, health system and societal value. The results of this exercise are illustrated below.

- Improved efficacy
- Increased tolerability
- Fewer or less severe side effects
- Enhanced patient convenience
- Improved compliance
- More highly targeted treatment
- Improved quality of life
  - Shorter disease duration
  - Prolonged symptom-free periods
  - Enhanced self-sufficiency
- Prolonged life expectancy

- Fewer diagnostic tests required
- Fewer physicians’ visits
- Reduced cost of medicines
  - Fewer high-end medicines used
  - Reduced need for add-on medicines
- Fewer emergency room visits
- Fewer surgical procedures
- Fewer and shorter hospitalisations leading to hospital bed closures

- Reduced absenteeism
- Greater productivity
- Reduced reliance on benefit systems (e.g., unemployment, disability, social security, pension)
- Reduced reliance on carers and increased self-sufficiency
- Improved quality of life
  - Increased happiness
  - Healthier, more active into old age
- Decreased social inequalities (e.g., access to care)
- Increased societal respect for elderly

- Industrial policy benefits
  - Capital investment
  - R&D workers employed
  - Taxes paid