PERSPECTIVES ON VALUE IN CANCER CARE
TABLE OF CONTENTS

About this concept paper 3
Executive Summary 4
Introduction 5
I. Value of innovation in cancer medicines 6
II. Many definitions of value 7
III. The development of “value frameworks” 9
IV. How is the patient voice integrated in value decisions? 11
V. How are other perspectives integrated? 12
VI. Short- and long-term views on value – a dilemma for policymakers 14
Conclusions & areas for further reflection 16
References 18
ABOUT THIS CONCEPT PAPER

This concept paper gathers perspectives and data around the issue of value in cancer care, with a focus on therapeutic innovation. The paper does not seek to be exhaustive nor suggest a single way of seeing, assessing and measuring value. To the contrary, this concept paper tries to map some of the most commonly cited perspectives on what makes cancer innovation valuable and to identify areas of difference as well as agreement among various stakeholders, with the intention to contribute to the wider policy debate on how to use value as a basic principle for organising cancer care systems and improving access to innovative cancer medicines.

This concept paper has been drafted on the basis of a literature review completed by research agency Rose Li & Associates Inc. in August 2016, an online perception survey of European oncology stakeholders performed by the Public Affairs consultancy Grayling, as well as insights from a multi-stakeholder group of experts. The experts involved include:

• **Dr Matti Aapro**, Dean of the Multidisciplinary Oncology Institute, Genolier, Switzerland; Coordinator of the Sharing Progress in Cancer Care programme at the European School of Oncology; Executive Board Member of the International Society for Geriatric Oncology

• **Natacha Bolaños**, Patients and Public Affairs Manager for the Spanish Cancer Patients Group and the Spanish Association for Lymphoma, Myeloma, and Leukaemia patients; Board member of the European Cancer Patient Coalition

• **Prof. Daniel Kelly**, Royal College of Nursing Chair of Nursing Research, Cardiff University; President, European Oncology Nursing Society (EONS)

• **Dr J. Gordon McVie**, M.D., Senior Consultant, European Institute of Oncology; Founding editor of ecancer.org

• **Andrew Oxtoby**, Vice President, Head of the International Oncology Business Unit, Eli Lilly and company

• **Prof. Kenneth R Paterson**, Fellow of the Royal College of Physicians (Glasgow, Edinburgh, London) FFPM; former chair of the Scottish Medicines Consortium (SPC); University of Glasgow

• **Dr Bettina Ryll**, MD/PhD; Founder, Melanoma Patient Network Europe

• **Prof. David Taylor**, Professor of pharmaceutical and Public Health Policy, University College London’s School of Pharmacy

Participation of the experts in the group is non-binding, voluntary and non-remunerated. Drafting and editing of the document as well as day-to-day implementation of the project is coordinated by Grayling. Eli Lilly & company (Lilly) is providing financial support for meeting costs and materials produced by the group. Lilly does not provide any fees to any of the members of the group for their involvement in this project. Lilly has provided comments on the document. The content of the final document reflects consensus from the members of the group who have full editorial control.
EXECUTIVE SUMMARY

More than one in four deaths in Europe are because of cancer; yet cancer expenditure accounts for just over 6% of overall health expenditure. Healthcare systems in Europe are trying to tackle this increasing cancer incidence by designing care pathways on the basis of what they believe can deliver the greatest value. However, there are currently wide variations in perceptions of what ‘value’ actually means amongst oncology stakeholders, which lead to different decisions on what is prioritised in terms of budget allocation. In this context, there is a need to clearly communicate and try to bridge these different views between cancer care stakeholders and decision-makers in Europe in order to foster effective policymaking.

This concept paper underlines the need for a workable framework for a wider discussion on the definition of value, taking into account three groups of elements:

1. Elements of clinical benefit e.g. overall survival, progression-free survival, response rates, quality of life/toxicity, etc.

2. Elements of societal benefit e.g. costs and savings for the healthcare system and/or for the patient, government-set priorities for budget allocation, return to work/life, etc.

3. External parameters such as time, geography, convenience, personal and societal preferences, that affect how the above two groups of elements are being assessed.

Linked to the above elements, is the inherent tension between the short term view and the long term view on value. Acknowledging that this is challenging for both governments with short term electoral cycles and health care systems with short term budget allocations, policy decisions and budget allocation choices should acknowledge this tension and take into account the long-term view on both 1) the often expanding value footprint of oncology medicines, and, 2) the long pharmaceutical R&D lifecycles which call for respectively long-term investment in the development of, and reward for, cancer medicines.

The paper underlines the need for the patient perspective to be appropriately taken into account when defining the benefits and wider value of cancer innovation. This should be done in a way that is meaningful for the healthcare system, respectful of the patient and with the goal of improving care and outcomes. It also acknowledges that there is a need for better understanding about how scientific progress in cancer treatment works, and the role of different types of innovation i.e. both breakthrough and continuous innovation, as critical parts of cancer care.

It has also become clear that further reflection and work is needed in a number of areas, namely:

• Mapping the definitions and methodologies used by healthcare systems in Europe to assess the value of oncology innovation, and to understand their differences, similarities and the different patient outcomes they lead to.

• Piloting outcomes-focused models using real-world evidence as a potential way forward for value-based care, under the right flexibilities and measurements.

• Incorporating elements of value, e.g. social value and savings for the healthcare system, which do not fit into traditional approaches and reimbursement decisions.

• Further collaboration and reflection in the fast-emerging area of value frameworks for innovative cancer treatments.

A new way of balancing short term budgetary decisions with long term interests in developing and integrating valuable innovation needs to be developed.
INTRODUCTION

More than one in four deaths in Europe are because of cancer, yet cancer expenditure accounts for just over 6% of health expenditure. The number of cancer diagnoses has increased by nearly a third between 1995 and 2012 and is expected to increase even further due to an ageing population and lifestyle factors. Cancer is set to become the biggest cause of death and disability in Europe with 17.3 million deaths globally, and has already overtaken cardiovascular disease as the main cause of death in 12 European countries.

Healthcare systems in Europe are trying to tackle this increasing cancer incidence trend by designing care pathways on the basis of what they believe can deliver the greatest value. However, there are currently wide variations in perceptions of what ‘value’ actually means amongst oncology stakeholders. These disparities are translated into different assessments of what is valuable in cancer care and therefore what gets prioritised in terms of budget allocation and what actually gets used within a care pathway or service.

There is therefore a need to clearly communicate and try to bridge these different views between cancer care stakeholders and decision-makers in Europe in order to foster effective policymaking. This concept paper does not seek to be exhaustive or to suggest a single way of seeing, assessing and measuring value. To the contrary, this paper will explore some of the most commonly cited perspectives on what makes cancer innovation valuable. The intention of this effort is to contribute to the wider policy debate on how to use value as a basic principle for organising cancer care systems and improving access to innovative cancer medicines.

The field of oncology care is wide and innovation has led to improvements in different areas of cancer care, including medicines, surgery, radiation, nursing and prevention. Efforts to assess value of innovation in these different areas have been undertaken and we should be looking at learning from each other. The main focus of this concept paper is on innovative pharmaceutical treatments and aims to contribute to the learnings, acknowledging that there is a need to engage in a wider discussion around the cancer care system and its different components.

Following a comprehensive literature review, a perception survey of policymakers and interviews with experts, we have found that the main areas of focus of this concept paper should be the following questions:

• What is innovation in oncology medicines?
• What makes an oncology innovation valuable? To whom?
• How are different stakeholder perspectives taken into account in decision-making?
• Are European healthcare systems integrating innovation deemed valuable?

This concept paper provides a number of conclusions and recommendations to European policymakers and oncology stakeholders with the aim of achieving a better understanding of what constitutes value in oncology innovation.
I. VALUE OF INNOVATION IN CANCER MEDICINES

Innovating is the process of translating an idea or invention into a good or service that is replicable and creates value by satisfying a specific need. Innovation in cancer care comes in many different forms: it can include processes, practices, products etc. Not every new idea qualifies as innovation. It must be implementable in practice and it needs to serve a need, therefore creating value for those benefiting from it. The close link between the notion of innovation and value is therefore evident.

Scientific progress in cancer care comes in many different forms: it can include processes, practices, products etc. Not every new idea qualifies as innovation. It must be implementable in practice and it needs to serve a need, therefore creating value for those benefiting from it. The close link between the notion of innovation and value is therefore evident.

Scientific progress in medical innovation, in cancer and beyond, has been taking the unmet patient need as a guiding principle, whether it comes to designing research and development programmes or in the effort of better understanding the biology of cancer. Outcomes of this progress have been met by varied levels of acknowledgment, depending on the level (and nature) of unmet patient need they have been considered to satisfy.

In the current environment of constrained financial resources, budget holders, and at times the wider public, tend to expect scientific progress to occur in great leaps. Once a cure becomes available, we often forget about the numerous steps it took to achieve that success. Scientific progress in cancer treatment is achieved in small steps and giant leaps, and the history of cancer innovation has often been one of continuous steps building upon one another. Societal preference leans towards ‘breakthroughs’, but given the nature of the disease these are less common than incremental benefits.

“Scientific progress in cancer treatment is achieved in small steps and giant leaps, and the history of cancer innovation has often been one of continuous steps building upon one another.”

Clearly, there is a need for both breakthrough and continuous innovation, as both are critical parts of cancer care. Continuous innovation (the sum of smaller and larger incremental benefits that continuously build upon each other) has in fact contributed to important benefits in cancer care. For example, today, more than 90% of men diagnosed with testicular cancer can be cured. It has taken 25 “steps” to achieve this level of progress but if one or more of these steps were not supported and used in clinical care the ‘next level of understanding’ of the disease may never have been unlocked for care to move on to the next ‘step’. It is critical to understand the role different forms of pharmaceutical innovation play, the needs they fulfill, and how we can best integrate them in the cancer care pathway.
Despite some clear views on how to define clinical benefit, a comprehensive literature review of available research has shown that ‘value’ is a term that is amenable to many different definitions. It has even been suggested that the ambiguity of the term is what has made it attractive.xiii Recognising this ambiguity, several stakeholders in the field of cancer care have published their own definitions reflecting their views on what constitutes value.

<table>
<thead>
<tr>
<th>ORGANISATION</th>
<th>YEAR</th>
<th>VALUE DEFINITION</th>
</tr>
</thead>
<tbody>
<tr>
<td>European Federation of Pharmaceutical Industries and Associations (EFPIA)</td>
<td>2016</td>
<td>Refers to Michael Porter’s definition “outcomes produced over the costs per patient”xix,xv</td>
</tr>
<tr>
<td>European Society for Medical Oncology (ESMO)</td>
<td>2015</td>
<td>The value of any new therapeutic strategy or treatment is determined by the magnitude of its clinical benefit balanced against its costxvi</td>
</tr>
<tr>
<td>European Observatory on Health Systems and Policies</td>
<td>2008</td>
<td>Value includes patient preferences, quality, equity, efficiency, and product acceptability among a wide range of stakeholdersxvii</td>
</tr>
<tr>
<td>National Institute for Health and Clinical Excellence (NICE)</td>
<td>2004</td>
<td>The value of a treatment is based on scientific value judgments, including a clinical evaluation and an economic evaluation, and social value judgmentsxix, including considerations of efficiency and effectivenessxix</td>
</tr>
</tbody>
</table>

These differing views also depend on which role is represented and what is seen as the most important element in one’s perception of value. Regulators look primarily at safety and efficacy, reimbursement agencies at clinical and cost effectiveness, and healthcare systems look at affordability. When it comes to a new treatment, regulators ask for a given set of data to assess whether the intervention carries benefit that outweighs the risks, while a payer may be looking for an additional set of evidence demonstrating superiority over existing treatments, cost-effectiveness etc.

The question from the perspective of reimbursement agencies and payers is not whether incremental and breakthrough innovations are equally important, but whether health systems should be asked to pay similar premiums for incremental innovation as they do for breakthrough innovation. This can lead to conflicting decisions, whereby the ‘marketing authorisation hurdle’ is overcome but the ‘reimbursement hurdle’ is not.

It should be noted that most regulators are not allowed to consider cost of treatment (focusing instead on the safety and efficacy) whereas reimbursement agencies are looking at effectiveness and cost. The two bodies may thus be looking at different data and may come to different decisions because their frameworks for decision-making are different. This is but one example of how different perspectives on value can lead to diverging, even conflicting, decisions.

Moreover, the issue can be further complicated due to the “perceived value” of new innovations. Potential health gains from some cancer treatments still in the research phase can be overstated in the media and may lead to a “hype” whereby everybody wants to use it and patients ask for it, regardless of whether it is the right option for them.

There is a general consensus on the fact that value is individualised – every stakeholder perceives a different meaning. For example, for a patient, value can be a few more months of life in good quality, whereas for physicians it can be seen as the benefit / risk ratio, and for payers it constitutes the maximum gain for a set amount of money. It has also been indicated that one’s perspective on value depends on the different stages of the disease, the tumour type and the individual’s options.

Whilst individual components can and will be different, depending on the exact disease, the overall definition
of value should be consistent and disease-neutral. Therefore, it has been suggested that one could look at value as a combination of three groups of elements:

1. **Elements of clinical benefit** such as overall survival, progression-free survival, response rates and quality of life/toxicity.

2. **Elements of societal benefit** such as costs and savings for the healthcare system and/or for the patient, government-set priorities for budget allocation, return to work/life and savings in other parts of the budget such as social benefits.

3. **External parameters** such as time, geography, convenience, personal and societal preferences affect how the above two groups of elements are being assessed in different cases. This means that if even if all the elements of the above two groups are identical, we may still see diverging decisions under different parameters.

Linked to the above elements, is the inherent difference between the short-term, static perception and the long-term, dynamic perception of value.¹ This discussion includes two very different topics: the one relates to the often expanding value footprint of oncology medicines,²³ and the other to the investment cycles linked to the R&D lifecycle of cancer medicines. At this point, we will look at the first of the two topics. The challenge here is that value assessments conducted by payers and HTA authorities often designed to draw from the data at hand and the expected, short-term benefit they could produce. Unfortunately, the economics of areas like anti-cancer treatment are complicated by the fact that such medicines are normally first used alone to treat late stage disease. Yet their optimum ‘cost per QALY’ value is likely to stem from using them with other drugs in earlier stage therapy.²³

Research has shown that cancer medicines’ full value only becomes apparent after they have been used widely in practice, in earlier lines, other tumour types or in combination with other treatments; and often after they have become low cost generic products. For example, the Office for Health Economics took ten commonly used cancer medicines²⁴ and found that seven out of these ten had additional value expansions following their initial indication. This ‘expansion’ of the value of an innovative cancer medicine is not captured at the point of the initial health technology assessment and pricing negotiations. End-of-life criteria might also impact the value that is attached to the assessment. Therefore, evaluating drugs only on the basis of their value at launch may fail to capture their importance for future developments.

¹ In the scope of this paper, ‘short term’ should be understood as what provides value for cancer patients in 2017, ‘long term’ should be understood as what provides value for cancer patients over the next quarter century.
III. THE DEVELOPMENT OF “VALUE FRAMEWORKS”

Recognising the need for simplification in the way value is being looked at, several organisations in the cancer care field have developed tools aiming to quantify benefit in cancer treatment. These tools, often called ‘value frameworks’ are usually based on a limited number of indicators but take into account additional complexities by allowing “bonus” points to be assigned for gains that are not well captured by primary metrics. Comparing these frameworks with each other and with Health Technology Assessment (HTA) authorities’ methodologies can provide useful insights into how payers, providers, and the public conceptualise treatment value.

There has been considerable literature looking at the differences and similarities of these value frameworks. Instead, in order to capture in a succinct and visual way the ‘weight’ that some of the most cited value frameworks and HTA authorities’ methodologies place on the different components of value, figure 1 below estimate how these fare in comparison to a societal average of the most often used indicators, or elements, of value.

![Figure 1](image-url)

What is evident from the above comparison is that all frameworks and HTA methodologies tend to undervalue components such as dignity, mental health, and the ability to continue working compared to how society values these factors. This lack of emphasis is at least partly due to the limitations of the available data: many frameworks rely exclusively on clinical trial data, and even when taking a broader perspective, it is challenging to compare across treatments and populations in a standardised manner.

Most assessments rely on data that compare new treatments to existing standards of care. This provides
important contextual insight into the value of a treatment in the current health care context. However, value assessments could differ substantially depending on the comparison chosen. Data from clinical trials may also fail to adequately distinguish between patient subgroups, potentially leading to underestimates of treatment benefit for some groups.

Moreover, a common question in these value frameworks is whether they should include a reference to the costs of the treatments they are evaluating. The American Society of Clinical Oncology (ASCO) has chosen to include treatment costs while the European Society of Medical Oncology (ESMO) has decided to not do so and instead focus on clinical benefit. Incorporating treatment costs or wider budget allocation concerns in a value framework poses several concerns. Firstly, it changes the focus from the ‘elements of clinical benefit’ examined above to also include the ‘elements of societal benefit’ and, to an extent, the external parameters. As we have seen, external parameters are hard to address in a set framework, given they change in relation to geography, time, the patient’s preferences etc. Even elements of societal benefit are often difficult to pin down.

For example, when introducing an innovative treatment to the cancer care pathway the costs to the health care systems are not limited to this specific medicine’s price. Costs associated with the fact that the specific disease was previously left untreated increases the overall cost of care for the system. At the same time, savings for the care system and the economy at large from such an innovative treatment are very often left out of the reimbursement decision, including a reduction in hospitalisations or complications from a new option that allows for home care, return to work and to normal life, or the simple fact of patients living longer and lead better quality lives. As long as this kind of data is not incorporated into value assessments, they will remain scarce and of low quality.

Value frameworks in cancer are an encouraging and welcome step in the direction of rational decision-making. For these frameworks to support decision-making, it is critical to clarify the goals and the scope of the framework itself. A tool aiming to help patients make a decision based on their personal preferences and clinical benefit (such as the ASCO tool) will look very different to a tool aiming to quantify clinical benefit alone to help decision-making for a treating physician (as the ESMO tools aims to do). These two types of tools will both be different from the tool a policymaker/payer will use to allocate budgets across therapeutic areas and within the cancer budget, which will be looking at elements of societal value, including cost of care, and external parameters that are not covered by the intent and methodology of the previous two.
IV. HOW IS THE PATIENT VOICE INTEGRATED IN VALUE DECISIONS?

An area presenting considerable room for improvement is how the patient voice is expressed and taken into account in value, HTA and reimbursement value decisions.\textsuperscript{xxvii} It is accepted that the patient voice is an important component in the definition of value of a new cancer treatment at any point in time: when deciding an R&D programme and designing a trial, when the regulator is deciding on a marketing authorisation application or when a payer is deciding to reimburse or not a new treatment, using the insights of an HTA process. The central role the patient voice plays in all these decisions is linked back to the first concept discussed by this paper: for a new idea, process or product to be considered an innovation, it has to fulfil a need. For a patient, what matters is the degree to which the medicine makes a tangible (positive) difference for them and the nature of that benefit.\textsuperscript{xxviii}

Better integration of patient perspectives in all efforts remains a stated goal of many stakeholders across Europe, and HTA bodies are getting better at incorporating patients' voices. However, implementation into routine practice differs across countries and regions.\textsuperscript{xxix} There are multiple reasons\textsuperscript{xxx} for which the integration of patient perspectives has not been fully translated into widespread routine practice:

- a decision-making process that does not provide for a formal procedural step to gather patient input;
- a check-box exercise with insufficient explanation on how the patient input is taken into account;
- a patient representative that has not been equipped with the necessary scientific and technical knowledge to provide his/her contribution in a way that it is understood by or helpful for the decision-making body.

There is a general agreement that patient perspectives on treatment value are highly heterogeneous. Individual preferences may be influenced by age, type and stage of cancer, treatment history, as well as personality and individual risk assessments. In fact, treatment value is likely to change considerably over the course of an individual's lifetime. Patient education and empowerment is key, both at the national and European level. Health literacy for patients is a key focus for many European patient groups and patients are generally better informed today compared to 10 years ago. There are a number of initiatives aimed at increasing the capacity of patients to better understand and contribute to medicines' R&D, regulatory approval and reimbursement.

There is a disagreement around the level of impact a patient perspective can carry in policymaking, depending on the content of the input (i.e. is it evidence based? What does it add beyond clinical data?), the transparency of decision-making, the level of expertise of the patient representatives and the time it is delivered. In the US, patient representatives active at FDA committees etc. have shown to be rigorous enough to critically question scientists and the pharmaceutical companies. Some experts indicated that policy and decision-makers do not always possess the full body of research available that outlines patients' perspectives.
V. HOW ARE OTHER PERSPECTIVES INTEGRATED?

Discussing value from a clinician or patient perspective means that their views should take prime position. However, when it comes to a definition of value used by the healthcare system, the policymaker or the payer, there are additional perspectives to consider.

In many European healthcare systems, in particular in countries such as the UK, Sweden, Denmark, Finland, and to a large extent, Spain, Portugal and Italy, a great part of the expenditure on healthcare is financed via general taxation rather than via contributions raised through taxes on wages. It is therefore important for the decisions (e.g. budget allocation, ways of organising the treatment pathway) of these actors to reflect the interests of the tax-payer and the wider society they seek to represent. That includes healthy members of the wider public who will never be diagnosed with cancer, members of the society who may be diagnosed in the future, and caregivers, who are both directly and indirectly impacted by the outcomes of cancer treatments.

Figure 2 below is based on a literature review looking at how different members of the wider public are looking into some of the main elements of value examined by payers, mirroring figure 1.

According to the figure, individuals who do not have cancer are likely to worry about the system-wide costs of care. They also assign high value to prevention and detection efforts, as well as long-term research and development that may lead to better treatments in the future.

Individuals who care for family members with cancer have a much higher stake in cancer treatments. As such, they are likely to take a broad perspective, valuing treatment efficacy and quality-of-life measures while maintaining an interest in detection, prevention, and innovation. Value assessments of individual caregivers are also likely to vary depending on the severity of illness of their loved ones.
Another point to note here is that it seems that as patients move through treatment and approach the end of life, the timescale of interest shifts from years, to months, to immediate needs. It is important to remember that many ‘cancer patients’ are now cured, or at least have their cancer modified to a ‘chronic disease’. They want the best cancer care but also realise that they personally may need non-cancer care. This can present unique challenges for payers, who must measure and address the needs of all groups within a single budgetary timeframe, but may also present opportunities to use innovative reimbursement approaches to create value across the lifespan.

In order for the general public to make appropriate health decisions and act on them, they must be able to locate health information, evaluate the information for credibility and quality, as well as analyze the risks and benefits. Investment in health education can enable all citizens, not only patients, to better understand their health condition and participate in the decision-making process to plan and manage their own healthcare plans, which can result in better outcomes. NICE’s Citizens Council could be a good example of how reimbursement agencies are incorporating the views of the public into their thinking.
VI. SHORT- AND LONG-TERM VIEWS ON VALUE – A DILEMMA FOR POLICYMAKERS

There seems to be consensus that cancer is a priority among most healthcare systems in Europe. However, the delivery of cancer care varies greatly between countries. Although survival rates are improving in every European country, there are still significant inequalities. Comparable data on cancer survival for European countries compiled by the European Cancer Registry (EUROCARE) has shown a general pattern of wealthier countries recording higher survival rates than poorer countries in Europe. Whilst the average 5-year relative survival rate for all cancer types combined was 54% in Europe for cancers diagnosed between 2000 and 2007, these rates varied from 40% in Bulgaria to 64% in Sweden.

Cancer survival is influenced by a range of factors, but wealthier countries often report better outcomes, also due to the fact that they record higher usage of newer cancer treatments. Variations in access to appropriate diagnosis, care and treatment, combined with inequalities in survival, suggest that not all people are accessing the best quality of treatment and care.

Cost containment and sustainable financing are among the biggest issues that are confronting healthcare systems and allocating resources to where they are most cost-effective is becoming an economic imperative. The question then becomes how we can continue to drive the same level of public and private investment in the research and development of innovative cancer medicines that will hopefully be the cures of the future. Investing in the future entails an understanding of the long-term value of innovation and the (necessarily) long time-span of investment. For investments in innovative medicines in particular, lifecycles are long and risk is high. It is therefore essential to consider that taking the short-term vision today and not rewarding innovation could mean that pharmaceutical R&D, and potentially the next cancer cures, are no longer an attractive option for investors. Well-structured policies could and should avoid such situations.

Some say that budget holders should pay for innovation once they have seen tangible outcomes. As healthcare systems try to shift towards value-based care, these types of arrangements (so-called “outcomes-based schemes”) are becoming more common in Europe. Outcomes-based schemes offer a way forward for value-based care, provided they set the right measurements and a process for revisiting financial agreements based on the performance: both upwards and downwards. Tying incentives and payment to outcomes is not just appropriate for cancer medicines but for healthcare systems as a whole, and may be better suited to address
the clinical, economic and affordability challenges of today. It is also a more transparent way of pricing and rewarding medical services.

In Sweden, for example, the Dental and Pharmaceutical Benefits Agency (TLV) evaluates cost effectiveness, decides on reimbursement status and sets prices for prescription drugs used in outpatient settings. In cases where drugs are granted conditional reimbursement, manufacturers may be asked to collect additional real-world data and to resubmit the cost-effectiveness model for evaluation by the TLV. In the United Kingdom, the National Institute for Health and Care Excellence (NICE)’s Managed Access Agreements (MAAs) are an example of how reimbursement agencies are developing schemes to grant conditional reimbursement, collecting additional real-world data, and reassessing it afterwards.

Whilst creating huge opportunities, it is recognised that the implementation of these schemes also poses significant challenges. Firstly, there is a fundamental need to establish appropriate infrastructure to capture, codify, understand and assess the data. Ongoing efforts to move towards payment schemes based on real-life use of medicines have often been halted by the lack of technological infrastructure, human and financial resources, the bureaucratic hurdles associated with the establishment of these tools, and delays in the timeliness of assessing the data once it is available. Secondly, data privacy is an ongoing concern, as compliance with existing data protection legislations ensuring robust mechanisms to protect patient privacy is critical. Lastly, analysis of the data is a challenge in its own right; independent third party mechanisms need to be established to fully integrate and analyse these datasets in order to generate conclusions which can contribute to regulatory- and reimbursement-related decision making. All these issues need to be discussed in a multi-stakeholder way and solutions to be piloted, before we can move to a system based on real-world experience.

The debate around how best to achieve long-term value in healthcare in Europe is not new. However, it is now critical to achieve consensus and find concrete solutions that can actually help healthcare systems deliver increasingly efficient and valuable care with reduced resources.”
CONCLUSIONS & AREAS FOR FURTHER REFLECTION

As evidenced by this paper, there are currently wide variations in perceptions of what ‘value’ actually is amongst oncology stakeholders. These disparities often translate into different, sometimes diverging decisions on what makes an innovative cancer treatment valuable and therefore what patients can have access to. There is therefore a need to clearly communicate and try to bridge these different views between cancer care stakeholders and decision-makers in Europe in order to foster effective policymaking.

This concept paper underlines the need for a workable framework for a wider discussion on the definition of value, taking into account three groups of elements:

1. **Elements of clinical benefit** such as overall survival, progression-free survival, response rates and quality of life/toxicity.

2. **Elements of societal benefit** such as costs and savings for the healthcare system and/or for the patient, government-set priorities for budget allocation, return to work/life and savings in other parts of the budget such as social benefits.

3. **External parameters** such as time, geography, personal preferences, convenience, and societal preferences affect how the above two groups of these elements are being assessed in different cases. This means that if even if all the elements of the above two groups are identical, we may still see diverging decisions under different parameters.

Linked to the above elements is the inherent tension between the short-term view and the long-term view on value. Policy decisions and budget allocation choices should acknowledge this tension and take into account the long-term view on both 1) the often expanding value footprint of oncology medicines, and, 2) the long pharmaceutical R&D lifecycles which call for long-term investment in the development of and reward for cancer medicines. Health Technology Assessment bodies cannot make short-term decisions without having a longer term view about the development of this area.

The paper underlines the need for the patient perspective to be appropriately taken into account when defining the benefits and wider value of cancer innovation. This should be done in a way that is meaningful for the healthcare system, respectful of patients’ views and their heterogeneity, with the goal of improving care and outcomes always in mind. It also acknowledges that there is a need to better understand how scientific progress in cancer treatment works and the role of different types of innovation, that is, both breakthrough and continuous innovation, as critical parts of cancer care.

It has also become clear that further reflection and work is needed in a number of areas, namely:

- A full mapping of definitions and methodologies used by healthcare systems in Europe to assess the value of oncology innovation is required. This exercise should focus on identifying differences, similarities and the
outcomes each system is producing. This is necessary before making any recommendations on which value assessment methodology leads to better outcomes.

• More flexible, outcomes-focused models using real-world evidence offer a way forward for value-based care, provided they set the right measurements and a process for revisiting financial agreements based on performance. However, significant challenges exist when it comes to putting this idea in practice. All these issues need to be discussed in a multi-stakeholder way and solutions to be piloted, before we can move to a system based on real-world experience. It would be advisable to develop a comparative analysis of the outcome-based models being piloted across Europe that is initiated now and will be completed once a number of the approaches currently being piloted in Europe have made their final recommendations.

• Further reflection is needed in the area of identifying and incorporating societal perspectives into decision-making. There have been a number of attempts to do so across Europe, but they generally run into practical challenges around incorporating elements of value that do not fit into traditional health economic modelling approaches. Ideally, decisions on budget allocation should carry a link to societal preferences, given funding of most healthcare systems in Europe is primarily based on the contributions of the wider society.

• The appearance of tools and frameworks proposing methodologies for assessing the value of innovative cancer treatments is an encouraging and welcome step in the direction of rationalising decision-making. For these frameworks to be able to help decision-making, it is critical to clarify the goals and the scope of the framework. Further collaboration and reflection is needed in this area.

• A new way of addressing short-term budgetary constraints with long-term interests in developing and integrating valuable innovation should be devised through once again a multistakeholder approach. This approach should align pipeline forecasting of new medicines entering the market with healthcare budget planning, to ensure that appropriate resources are allocated at the right time.
REFERENCES


Bengt Jönsson et al., *Comparator Report on Patient Access to Cancer Medicines in Europe Revisited*, 2016 – page iv: the direct health cost of cancer has remained more or less flat around 6% of total health expenditure over the last 20 years.


Bengt Jönsson et al., *Comparator Report on Patient Access to Cancer Medicines in Europe Revisited*, 2016 – page iv: the direct health cost of cancer has remained more or less flat around 6% of total health expenditure over the last 20 years.


Bengt Jönsson et al., *Comparator Report on Patient Access to Cancer Medicines in Europe Revisited*, 2016 – page 4: The elderly account for a growing share of the total population. It is clear that the risk of getting cancer increases at old age, and thus a growing share of elderly gives rise to more cancer cases. External references: Eurostat. Population on 1 January by five years age group and sex [demo_pjangroup] [January 22, 2016]. Available from: http://ec.europa.eu/eurostat/


http://www.estro.org/about/health-economics-in-radiation-oncology---hero/hero


National Cancer Institute. Testicular Cancer Treatment (PDQ®): General Information About Testicular Cancer Available at: http://www.cancer.gov/cancertopics/pdq/treatment/testicular/HealthProfessional


http://www.efpia.eu/topics/innovation/outcomes

http://www.esmo.org/Policy/Magnitude-of-Clinical-Benefit-Scale/Article


http://eprints.whiterose.ac.uk/134/1/culyera1.pdf

Rejón-Parrilla JC, Karla Hernández-Villafuerte, Koonal Shah, Jorge Mestre-Ferrandiz, Louis Garrison, Adrian Towe, The Expanding Value Footprint of Oncology Treatments, poster presented at ISPOR 2014

Rejón-Parrilla JC et al, The Expanding Value Footprint of Oncology Treatments, poster presented at ISPOR 2014

Rejón-Parrilla JCat el, The Expanding Value Footprint of Oncology Treatments, poster presented at ISPOR 2014

Neon Brooks, Silvia Paddock, and Samuel Thomas, Rose Li and Associates, Inc Discussion Paper on Perspectives on Value in Cancer Treatment [2016, on file]


Cherny et al. (2015) *A standardised, generic, validated approach to stratify the magnitude of clinical benefit that can be anticipated from anti-cancer therapies: the European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS)*, Annals of Oncology [2015] 26 (8): 1547-1573 [https://m.annonc.oxfordjournals.org/content/26/8/1547.abstract]


xxx https://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Public-involvement-programme/patient-involvement-ta-process.pdf


xxvii Bengt Jönsson et al., Comparator Report on Patient Access to Cancer Medicines in Europe Revisited, 2016 – page 61: Between 1990 and 2007 there was a steady increase in cancer survival rates in all countries, yet disparities remain between wealthier countries with higher survival rates and poorer countries with lower survival rates

xxviii Bengt Jönsson et al., Comparator Report on Patient Access to Cancer Medicines in Europe Revisited, 2016 – page 144: Low national income and health care spending per capita are major obstacles for access to new cancer drugs

xxix Bengt Jönsson et al., Comparator Report on Patient Access to Cancer Medicines in Europe Revisited, 2016 – page 141: Countries in Eastern and Southern Europe, with low GDP per capita, have sales at around 1/3 of sales in countries in Western Europe, both in 2005 and in 2014

xxxi Bengt Jönsson et al., Comparator Report on Patient Access to Cancer Medicines in Europe Revisited, 2016 – page 61: Between 1990 and 2007 there was a steady increase in cancer survival rates in all countries, yet disparities remain between wealthier and poorer countries

xxxii Taylor D, Affording the Future? The role of cost effectiveness thresholds in determining NHS patient access to high quality care in the post-Brexit era, 2016

xxviii http://www.eurocare.it/Results/tabid/79/Default.aspx#EuroProsp