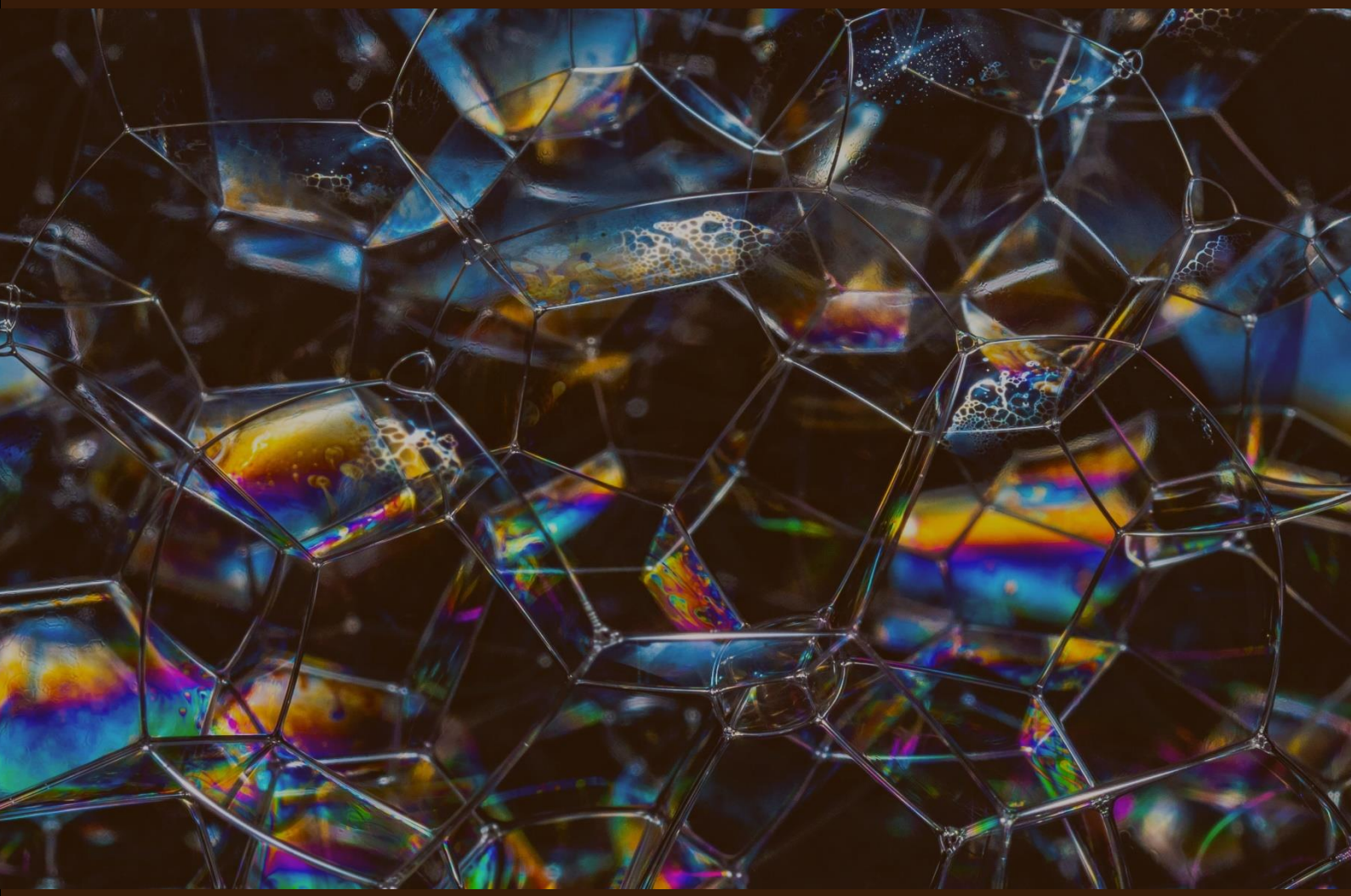
executive insight
HEALTHCARE CONSULTANTS



Healthcare innovation:

Patient access friend or foe?

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Healthcare innovation: Patient access friend or foe?

Increasing innovation in healthcare may be complicating the communication between manufacturers and payers and may be creating additional patient access issues.

By Mary Rose Ropner – Development innovation in its various forms is driving oncology medicines to become ever more technologically complex and advanced. In some cancers, there has been significant progress from the first medicinal solution to cancer – chemotherapy – towards ever more effective and personalized treatments, such as CAR T therapy.

However, this drive towards innovation and better outcomes isn't just making the treatments themselves more complex, it is also increasing complexity around every part of the product lifecycle, including around how payers need to assess the product to approve or refuse access. This is creating further communication challenges which need to be addressed now to prevent a decrease in trust between manufacturer and payer which may be detrimental for patient access to new drugs.

Development innovation drives complexity

Novel trial designs (e.g. basket, umbrella, and platform) are being used to ensure that the efficacy of drugs is accurately measured, but they are more difficult to interpret because they can allow drugs to be assessed for ten different cancers all at once, instead of only one. This means that a single trial can include patients with many different types of cancer all receiving a single treatment, making it more complicated to understand how the experimental product compares against what is typically used to treat whichever cancer the patient in the trial may have.

Novel trial designs may include patients with many different types of cancer, which makes it more complicated to understand the added value and the true efficacy.

Heterogeneity is also being identified more frequently within specific cancers as we learn more about the disease mechanisms, meaning that where you previously had a single cancer (e.g. breast cancer), you now have potentially 3 or more (e.g. HER2+ breast cancer, triple negative breast cancer, ER+ breast cancer, etc.). When drugs for these specific sub-types finally make it to market, it can be more difficult for a healthcare system to identify these patients correctly due to costly diagnostic procedures and tests, and the potential for false positives increases as the incidence of the population decreases.

Additionally, as more drugs are developed for specific cancer sub-types, it becomes more difficult to conduct a clinical trial to assess their efficacy, as there are fewer patients available to enroll. The application of additional inclusion or exclusion criteria may narrow the treatable population further still and reduce its applicability to the general population in a given market or region.

Will a new drug work in a real-world setting?

Finally, if these drugs are perceived as transformative at early stages of development they may achieve market approval with extremely immature data, which makes it more difficult still to understand how well a patient will actually respond in the real-world setting to the treatment.

All of these challenges are directly causing issues for health technology assessment (HTA) agencies such as the National Institute for Health and Clinical Excellence (NICE) in England, the Scottish Medicines Agency (SMA) in Scotland and for other payers in markets across the globe.

Complexity is creating new challenges for payers

While many of these issues are impacting payers across the globe, they are causing specific additional complexities for the UK's NICE & NHS England (NHSE). These were shared by NICE along with additional information on how complexity was affecting their reimbursement

assessment decision making at a conference in London, UK, March 2019.

Many highly innovative products are accepted by the European Medicines Agency in the European Union (EU) and arrive at NICE's doorstep with a conditional approval based on immature data and limited information to show that the drug will work in the UK's population and/or whether it will work in all patients in whom it is administered. This is often due to the fact that these drugs are approved based on a single arm trial instead of a comparative randomized clinical trial. As NICE is a cost-effectiveness focused payer, these questions are particularly important to ensure that its budget is distributed appropriately but are often unaddressed in submission dossiers forcing decisions to potentially be made despite the lack of appropriate evidence.

Highly innovative products face additional hurdles when it comes to the needed approval by payers.

NICE may also be faced with a decision over how it should assess the technology, which may be highly innovative, difficult to evaluate and highly uncertain (e.g. CAR T), as well as needing to make a final reimbursement decision for the product.

Payers across the globe are also experiencing increasing pressure from public bodies such as charities and patient organizations over reimbursement decisions – especially for high value products for rare diseases with small populations which may be refused due to their cost. Many will lobby against a negative reimbursement decision, on

the basis that a new product may still help some people who now will be denied treatment. In England, a negative reimbursement decision is only reached after thorough assessment of the evidence provided, and calculation of cost-effectiveness – a process which is facilitated by the provision of additional longer term data and hindered by submissions containing high levels of uncertainty. To patients, however, this can simply be viewed as unnecessary restriction of access based on price which merely directly hurts patients, making the "patient voice" increasingly powerful.

Negative reimbursement decisions and the increasingly powerful voice of patients

Manufacturers need to partner with payers to ensure fast and sustainable patient access

All of these challenges and issues have the potential to erode the trust between payers and manufacturers and obscure the common goal between the two: getting the best treatments to patients under acceptable conditions (e.g. at a cost-effective price). The solution lies in improving understanding around each other's priorities and goals. The potential is that, by doing this, additional commonalities will be revealed and the process of getting these products to patients can be expedited rather than hindered. Submission dossiers should be tailored to ensure that they meet payer needs, which can differ from market to market. For example, NICE requires manufacturers to provide multiple cost-effectiveness analyses, while the Institute for

Quality and Efficiency in Health Care (IQWiG) and Federal Joint Committee (GBA) in Germany are highly interested in the clear demonstration of the clinical effectiveness of the product and the justification around the choice of appropriate comparator therapy.

At present, it is possible that manufacturers and payers may each perceive the other in a different way, and these perceptions may be misaligned with reality, creating an additional layer of complexity in the communication process. This needs to be addressed before the two can move forward and work together effectively. Manufacturers should not view payers as an obstacle to overcome, but as a partner with which they can work with to achieve success and potentially reform when required. These institutions were created to ensure that effective and safe drugs get to their citizens as quickly as possible, an aim which manufacturers can proactively support by providing relevant, implementable solutions such as managed entry agreements (MEAs), real-world evidence (RWE) collection programs or solutions to support supply issues or infrastructure concerns at the earliest interaction.

Manufacturers and payers need to work together to achieve a common goal: bring safe, effective drugs to market, fast and at a fair price.

The way forward

Collaboration between manufacturers and payers will be key in ensuring that patients have access to future innovation, e.g. high-cost oncology combinations. Trade associations should aim to work with payers to achieve an open two-way dialogue to address any issues identified and steps are already being taken to get there. One example is that in the UK, the 2019 Voluntary Scheme for Branded Medicines Pricing and Access has been agreed. This is a non-contractual voluntary arrangement between the UK's Department of Health and Social Care (DHSC) and the Association of British Pharmaceutical Industry (the representative body for the pharmaceutical industry in the UK, ABPI). It includes a statement indicating that the DHSC and NHSE will provide feedback on ABPI's proposed solutions to allow company-to-company engagement as a way to ensure that high-cost combination therapies can be developed for and successful within the current NICE appraisal process.

While there may be many examples of countries and manufacturers taking small steps, there is still much to do to improve the relationship between these two key stakeholders, but it is vital that this is achieved to ensure that patients will continue to get access to the best care possible whenever health issues arise.

Only if both sides collaborate and communicate in the right way, will patients get access to the best care possible.

About Executive Insight

Experienced Healthcare Consultants

Executive Insight is a specialized healthcare consulting firm which supports biopharmaceutical companies to successfully prepare, launch and commercialize their products. Since its inception 20 years ago, it has supported the design, co-creation and implementation of collaborative approaches between manufacturers and the pharmaceutical industry with the aim of improving the quality of engagements and overall cross-party understanding.

Executive Insight has extensive experience in conducting innovative strategic projects and continues to strive to develop the thinking in the healthcare industry, including in understanding how to address new challenges such as how companies should partner with payers to fund novel products like CAR T or eliminate infections such as hepatitis C.

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