Written evidence submitted by Takeda UK Ltd (CSV0048)

Takeda UK Ltd is fully supportive of the Keep Up With Cancer's response to the Committee's inquiry, including their position on the limited availability of treatments in England and lack of flexibility in medicines pricing as factors behind the difference in cancer outcomes in England and comparable countries internationally. Takeda UK Ltd endorses Keep Up With Cancer's response on meeting the ambitions of the Long Term Plan for cancer and the need for a new cancer strategy for England. Keep Up with Cancer comprises seven life sciences companies with a common interest in oncology developments in the UK.

Takeda UK Ltd would like to make some supplementary points to the Committee around the barriers to patient access to new advances in cancer treatment – specifically combination treatments and Advanced Therapy Medicinal Products (ATMPs) – and the collection and utilisation of data in cancer care, and the opportunity by addressing these to improve patient outcomes, both now and into the future.

The UK is a world leader in life sciences research. For example, the UK cell and gene therapy industry employs 1,500 people and by 2035, the cell and gene therapy industry could be worth £10 billion and provide 18,000 jobs[i]. The UK should be commended for its strong support for scientific innovation. However, given the complexity and relative novelty of new cancer treatment advances, there are challenges to ensuring that new cancer therapies reach patients in need and therefore an appropriate incentive for continued investment in the UK, particularly where technologies address a high unmet need in rare conditions.

The potential consequences for the UK's leadership position from barriers to patient access are evident from a recent example of a manufacturer of gene therapies for rare conditions disinvesting in operations in Europe, citing a difficult payer environment.

Combination treatments

Combination treatments are where two or more individual treatments – the backbone and add-on treatment – are combined together. The backbone treatment is a treatment or treatment combination that is already available to patients. The add-on treatment is a treatment or treatment combination, that is added to the existing backbone treatment.

We know from experience in complex diseases, such as cancer, HIV, rheumatoid arthritis and Hepatitis C, that combination treatments have the potential to deliver significant clinical benefits to patients. This is because using multiple treatments in combination can simultaneously target numerous pathways that drive a disease. As our understanding of complex diseases increases, combination treatments are becoming increasingly common to improve patient survival and quality of life.

Despite potentially delivering significant clinical benefits to patients, combination treatments often face cost effectiveness barriers even if the new add-on treatment were to be given away at zero price. Indeed, in 2019, only 56% of licensed combination treatments were routinely available to patients on the NHS, in the UK.[ii] Some of the most clinically effective cancer medicines are not available in England due to the combination medicines challenge (e.g. multiple myeloma therapies which are available in other comparable European countries)

There is consensus within key stakeholder groups, nationally and internationally, that solutions for accessing combination treatments need to be found quickly for the benefit of patient outcomes, now and in the future.

This solution must necessarily include the ability to allow for flexible, non-uniform pricing. The *NHS commercial framework for new medicines* states that the NHS in England will continue to adopt uniform pricing by medicine and that it does not operate blended pricing or pricing by indication except in specific and defined circumstances. Combination treatments represent an exceptional circumstance which can offer improved patient outcomes, and we strongly believe non-uniform pricing should be considered for medicines that fall within this situation.

Collection and utilisation of data in cancer care

Improved collection and utilisation of data in cancer care would allow the NHS to better understand the outcomes provided by a medicine for patients and ensure the NHS is fairly paying for treatments that deliver clinical value.

The health service should prioritise improving the scope, quality, completeness, and interoperability of the Systemic Anti-Cancer Therapy (SACT) dataset. To support enhanced clinical decision-making, all relevant bodies and industry should explore options to ensure collection of real-world evidence to be used to support flexible pricing schemes. Lastly, building on the COVID-19 response, all relevant bodies (HMT, NHSE, NHSX, NHS Digital, PHE) should work together to improve the collection, utilisation and linkage of clinical cancer outcomes datasets for the benefit of patients.

ATMPs

ATMPs represent a departure from traditional medicines, in so far as they involve the modification and implantation of living human genes, tissues and cells, rather than chemical or biological molecules, and therefore require a different approach for their entry into the NHS. The potential of ATMPs has driven the pharmaceutical industry to invest a growing share of its pipeline to bring these medicines to patients, such that we stand on the cusp of a wave of ATMPs entering the NHS.

1) Adapting Health Technology Assessments methodologies

To facilitate the entry of ATMPs into the NHS, NICE's Health Technology Assessment framework needs to adapt to better accommodate ATMPs.

With the potential for a long-lasting benefits but accompanied by an inherent challenge in demonstrating enduring benefits over the long term (although offering predictability of healthcare costs, minimizing uncertainty around affordability), ATMP clinical trials may necessarily depend on surrogate outcomes, requiring HTA bodies and payers to extrapolate to clinical endpoints. NICE should enhance acceptability of validated surrogate endpoints to estimate long-term outcomes and adopt accommodative changes in economic modelling such as improving methods for extrapolation for ATMPs.

Further, the need for greater acceptance of real-world evidence and the importance of these data to inform/supplement the evidence base for new medicines. Greater acceptance of non-RCT data is crucial, particularly in the context of earlier licensing decisions and likely immaturity of data for ATMPs. We would welcome clear guidance to generate, analyse and present real-word evidence.

Additionally, it can be difficult to identify the appropriate comparator for ATMPs to be assessed against, particularly where the ATMP therapy leads to marked changes in clinical practice or where there is no existing therapy. NICE should undertake further research to improve its methodology of indirect comparisons.

2) Managed access processes

While offering potentially transformative benefits over a long-time horizon, ATMPs can be associated with higher upfront costs, reflecting the value they bring to patients, healthcare systems and society at large. The NHS, however, is not generally configured to pay for new medicines in a manner other than a price per unit, whether by vial, treatment or procedure, leading to a divergence in the timing of product cost and the benefits it generates. This therefore can present a significant impact to healthcare budgets within a relatively short space of time.

Additionally, ATMPs can have significant uncertainty at the time of a product's launch. In some cases, it may be decades before the clinical benefit of an ATMP is known to be as transformative and enduring as first anticipated at the time of regulatory approval.

Collaboration between the Government, NHS and pharmaceutical industry to develop innovative and sustainable financial solutions is therefore required to overcome inherent affordability and uncertainty challenges to ensure patient access. Importantly, new proposals on commercial and managed access must work in conjunction with existing pathways including the Innovative Licensing and Access Pathway (ILAP) and the Innovative Medicines Fund (IMF).

3) Better evidence collection

To enable innovative payment models that address uncertainty, robust Real-World Evidence (RWE) infrastructure will be critical to support long-term evidence generation of the benefits of ATMPs in a clinical setting.

Currently, the ability to access data in a format which is analysable is difficult. There many datasets with different variables captured across the country, sometimes from individual hospitals or centres.

The NHS must make significant progress to improve the scale, interoperability and accessibility of health data, and ensure there is also a clear and consistent approach to data governance. To relieve the administrative burden to register patient outcome data, financial incentives for clinicians and providers may also need to be considered.

The workforce must be supported with training and education programmes to ensure the NHS is fully equipped with data science skills to fully exploit the opportunity of data generation.

4) Redesigning and optimising services

Some ATMPs are very complex, which may involve the use of special devices, specific clinical training and long-term follow-up patient monitoring. This can present unique challenges to services that are configured around the administration of more conventional therapies. If an ATMP is 'off the shelf' (i.e. allogeneic) and can be administered outside of tertiary centres of excellence, it will be imperative to ensure the necessary infrastructure is in place to support this in order to expand access to more patients.

The NHS should ensure there is effective horizon-scanning and forward planning ahead of the introduction of ATMPs. As timelines for ATMP commercialisation can change – owing to their innovative nature, focus on addressing unmet need and therefore being subject to accelerated regulatory approvals – it is also necessary to ensure flexibility to forward plan effectively. Horizon scanning should therefore factor in base case timings, which manufacturers are working towards, and best-case timings, premised upon regulatory approval after phase I and II data and/or an accelerated licensing pathway e.g. ILAP/ORBIS.

The NHS should continue to engage with through multi-stakeholder fora with industry, particularly the Accelerated Access Collaborative, to find solutions that span various areas of responsibilities.

5) Supporting clinical research

It is essential to support the further easing and speed of setting up clinical trials on the NHS to bridge the gap between scientific research and full-scale commercialisation of ATMPs in the UK. Effective clinical research infrastructure within the NHS will ensure that products progress effectively through clinical development and can be commercialised to reach UK patients.

[i] Alliance for Regenerative Medicine and the BioIndustry Association, Leading Innovation, The UK's ATMP Landscape, 2019. Available at: https://www.bioindustry.org/resource-listing/leading-innovation.html

[ii] EFPIA and IQVIA., EFPIA Patients W.A.I.T. Indicator 2019 Survey. EFPIA. 2020. Pg 49

Sept 2021