Optimising data collection to facilitate access to ATMPs

The purpose of this paper is to outline the ABPI's recommendations for key principles to optimise data collection for ATMPs in the UK. This paper has been developed with a focus on England, but recommendations could be useful for consideration across the devolved nations.

Executive summary

Now more than ever, the UK is focussed on the potential to further develop data and digital capabilities to improve health outcomes and the delivery of care. Recent policy developments such as the Genomics implementation plan, the DHSC's data strategy and NICE's five year strategy indicate strong potential for the life sciences sector [1] [2] [3]. Improved data capabilities will be essential for the UK to position itself as a leader in the field and to be prepared for the growing pipeline of ATMPs.

Ensuring the UK can continue to provide timely patient access to innovative medicines will also be essential to secure its position as a priority launch market. Innovative payment models such as outcomes-based and spread payments can help manage the reimbursement challenges that are often exacerbated for ATMPs, namely: affordability and uncertainty. However, for these to be considered by NICE and to be effectively implemented, they will need to be supported by robust, standardised national data collection practices and infrastructure.

The ABPI has considered challenges with the UK's current data capabilities and examples from other countries to produce eight key principles that can help towards realisation of its full data potential. These key principles include: robust data governance, avoid duplication, completeness & quality of data, timely access, national funding, a UK-wide plan, learn with international partners and assess impact.

1. **It is imperative senior government decision-makers, NHS, clinicians, academia and industry are aligned on the UKs future data strategy and communicate this with clear articulation of the potential benefits and data governance reassurance to the public.** If a significant proportion of patients opt out of NHS data collection, it will have negative implications on the quality and usability of data. There is opportunity for communications to build on Genomics England public engagement work in rare disease and oncology.
2. **NHS England, NHS Digital, NICE, MHRA and companies should engage early on data collection requirements to facilitate preparation for new medicine launches.** Early engagement mechanisms provide an opportunity for system partners and companies to discuss potential for additional data collection. Furthermore, the new Innovative Licensing and Access Pathway (ILAP) provides a mechanism for early engagement with key stakeholders and NHSE should participate in Target Development Profile (TDP) meetings for products that are potential candidates for managed access or outcomes-based pricing.

3. **NHS England, NHS Digital, NICE, MHRA and industry should work together to align on expectations for future data collection practices.** In the short-term, it is important for industry to be aware of new data collection requirements for ATMPs in the UK. For example, it is currently unclear whether these could be similar or duplicate to EMA registry requirements for ATMPs and could significantly impact UK launch planning. In the longer-term, system partners and industry should engage in discussions on expectations for data collection practices to evolve in the UK (e.g. a national database or a Trusted Research Environment (TRE)).
4. **NICE and NHS England should consider opportunities for the broader application of data collection beyond Managed Access Agreements (MAAs) for ATMPs.** The Accelerated Access Collaborative are already monitoring the ATMP pipeline to identify future launches that may benefit from MAAs and evaluating data collection capabilities in these disease areas. This work could also support consideration of outcomes-based payments, where appropriate.
5. **There is strong potential for Scotland or Wales to provide a proof of concept for establishing a national standardised data set that could be adopted across the UK.** This could take the form of exemplar pilots focused on a specific disease area, which could be identified from the ATMP pipeline.

Background

High quality data and digital systems are needed to better understand disease, support the development of new treatments, monitor the effectiveness of existing interventions, and support the planning and provision of care. The COVID-19 pandemic has shone a light on the importance of this and now more than ever, the UK is focussed on further developing data and digital capabilities to improve health outcomes and the delivery of care. There is already a considerable amount of activity ongoing in this space, which indicates strong potential and exciting opportunities for the life sciences sector. Recent policy developments include the Genomics Implementation plan to position the UK as the most advanced genomic health system in the world, the Department of Health and Social Care's (DHSC's) strategy to ensure the full potential of data is realised and the National Institute for Health and Care Excellence's (NICE's) five year strategy highlighting the importance of real-world evidence (RWE) and including leadership in data as one of their four key pillars [1] [2] [3]. Furthermore, the MHRA, NICE and Scottish Medicines Consortium (SMC) launched the new Innovative Licensing and Access Pathway (ILAP) in January 2021. The aim of ILAP is to accelerate time to market, facilitating patient access to innovative medicines which are granted an "Innovation Passport" [4]. The launch timing for ILAP has been ideal given the ongoing NICE methods and process review and announcement of a new Innovative Medicines Fund (IMF) by NHS England [5]. The availability of ILAP and the IMF increases potential for ATMPs to be reimbursed via

managed access agreement, which would generate real world evidence. The ABPI have previously published a report reviewing how industry uses health data to develop new medicines and provides proposals for industry to work with the Government and NHS to realise the potential of the UK's health data [6].

Opportunities for ATMPs in the UK

Improved data capabilities will be essential for the UK to position itself as a leader in the field of ATMPs, ensure patients have timely access to innovative treatments, to continue to be an attractive market for manufacturers to invest in R&D, and remain a priority for new treatment launches. However, a growing pipeline of ATMPs and the introduction of faster licensing approval routes is likely to exacerbate challenges HTA organisations and payers face when determining whether to reimburse and grant access to new treatments. Ensuring the UK can continue to be provide timely access to innovative medicines is also essential to o help develop the UK as a world leader for ATMPs.

ATMP payment model challenges

ABPI has previously produced a position paper outlining the need for payment models to help manage the reimbursement challenges, which can be broadly summarised as [7]:

- **Affordability**: ATMPs often have high upfront costs and growing pipeline increases need to manage the budgetary impact
- **Uncertainty**: challenges demonstrating the longer-term efficacy and durability at time of launch to meet HTA requirements

The two types of payment models recommended by this paper are outcomes-based payments and spread payments (e.g. over multiple years). However, for these to be considered by NICE and to be effectively implemented, they will need to be supported by robust, standardised national data collection practices and infrastructure.

UK experience with outcomes-based agreements

While the UK already has some experience with outcomes-based payments schemes, these are limited and not being routinely considered to support access to new medicines. For example, in 2015, NHS England introduced a 'pay if you clear' scheme, where the treatment cost for a new hepatitis C treatment will be refunded for patients that have not successfully cleared the virus after 12 weeks [8]. In 2016, NICE introduced the reformed Cancer Drugs Fund (CDF) to recommend medicines for temporary reimbursement with additional evidence collection for a defined period [9]. The CDF has data collection requirements, which could be considered a steppingstone towards outcomes-based payments, but it is limited to oncology. A new Innovative Medicines Fund (IMF) will enable this conditional approval approach to be routinely available for non-cancer medicines [10]. While the introduction of the IMF is a positive step to help enable early patient access to some non-cancer medicines, it is unlikely to be a

suitable option for many ATMPs given it is a mechanism to resolve short-term uncertainties. In addition, the CDF and IMF are ring-fenced funds with an annual budget which is unlikely to be sustainable for providing access to a number of ATMP's given their often-high upfront costs [11]. Therefore, decision-makers will also need to buy into the longer-term inherent risk associated with certain innovative treatments.

Challenges with current data collection practices in the UK

Gaining public trust

Data sharing remains a controversial topic, despite it's potential to help mange individual patient care, improve services, support diagnosis and treatment. Patient and public concern with data sharing is often related to privacy, particularly following high-profile NHS data security breaches [12]. In 2018, the NHS implemented a national data opt out for patients and to choose whether they wish for their identifiable, personal data to be used for research or planning purposes [13]. If a significant proportion of patients decide to opt out, this will negatively impact the quality and validity of available data [12] and will be particularly challenging for diseases with a low prevalence. Concerningly, a recent UK study on the public perception of data sharing found respondents to be less willing to share data when used for commercial purposes and indicates a decline in willingness compared to research from previous years [14]. A key challenge for collecting data for outcomes-based payments is often whether individual patients are potentially identifiable and whether data sources allow linkage between other sources.

Variation by disease area

There is currently no single data system in the UK that can collect data for all conditions in a consistent way that includes primary, secondary, tertiary and social care information and delivers high quality data outputs that can be used and re-used to deliver better patient outcomes. Infrastructure is already in place for routine collection of oncology data with the National Cancer Registration and Analysis Service (NCRAS), including the Systemic Anti-Cancer Treatment (SACT) database in England. However, SACT it is unlikely to be sufficient to support outcomes-based payments. A study evaluating the completeness of SACT data found the data fields already included in SACT with the strongest potential to support outcomes-based payments to have a completeness average of 58% [15]. The regimens outcome summary would be particularly important for outcomes-based payments and had an average completeness of 12%.

National data infrastructure is also already in place for many other (non-oncology) conditions, but research has highlighted considerable inconsistencies in whether these registries collect data by indication, has mandatory data collection requirements and the duration of data collection [16]. There is also a need for consideration of how to securely incorporate data from

wearables and other digital solutions that can help collect outcomes data and provide patients with feedback.

Inconsistencies in data collection practices

It is vital that data infrastructure is supported by sufficient data collection practices. However, research has found differences according to the care setting. For example, long-term data collection and monitoring is often part of routine clinical practice in specialist care for chronic and severe conditions [16], whereas long-term data is more challenging to collect for patients with acute and short-term conditions that are discharged to primary care [16]. The collection of data can lead to an increased administrative burden on clinicians. However, as technology evolves there is potential for patients to contribute to data collection through wearables.

Lack of connectivity to all relevant stakeholders

Research by the Cell and Gene Catapult has also demonstrated that even in indications where existing data collection infrastructure is most likely to be able to support outcomes-based payments (i.e. oncology, end-stage renal disease, haemophilia A and beta thalassemia), there remains a need to upgrade connectivity to relevant stakeholders [16]. This would require linking the dataset to clinicians, hospital pharmacists and commissioners. The manufacturer that has entered the outcomes-based agreement would also need visibility of relevant data. Patients should also be able to access their data if requested. Furthermore, access should be secure and enable stakeholders to administer the data elements of a contract without creating additional burden on the data holder.

Learnings from other countries

Similar to the UK, other leading European pharmaceutical markets have experience with agreements based on a medicinal products performance or outcomes. However, unlike the UK, some of these countries are more frequently engaging in these types of agreements and have established national datasets to facilitate their implementation. While outcomes-based agreements are more complex to design and implement than a simple discount, they are a useful mechanism in certain complex circumstances. Particularly, when there is a high unmet need for a new medicine, the available evidence is immature and significant uncertainties are not able to be addressed in the short term. The need to manage such uncertainties will be exacerbated with the UK offering more accelerated licensing routes and the growing ATMP pipeline.

The Italian Medicines Agency Web Registries

Italy was one of the earliest adopters of outcomes-based agreements given the Italian Medicines Agency (AIFA) has been implementing prescription monitoring since 2005 [17]. Today, a range of different types of Managed Entry Agreements (MEAs) can be routinely

considered to support access to new medicines in Italy due to AIFA's national web-based registries [18]. These registries enable every prescription for innovative and high-cost medicines to be tracked by requiring all hospital consultants to complete a form that validates the prescription and automatically requests the medicine from the hospital pharmacy [19]. Challenges with the system have been highlighted and can provide useful learnings, such as the rate of updating the forms from clinicians and delays in requesting refunds from pharmaceutical companies [20]. AIFA has since introduced a new 'payment at results' model, to enable deferred payments linked to performance [21]. This new model was introduced to support the reimbursement of a novel CAR-T therapy, and while it removes the need to request a refund, it requires upfront agreement on the outcomes to be measured and performance expectations.

Spain: Valtermed Registry

In 2019, the Spanish NHS introduced a new data collection and management system called Valtermed to enable payment-for-results agreements for seven new innovative treatments, including CAR T-cell therapies [22]. All medicines tracked by Valtermed have a pharmaco-clinical protocol that is developed by a multidisciplinary group of experts. It outlines criteria for what outcomes to measure, when to measure them and defines the therapeutic objective of each measure [22]. Further expansion of the Valtermed system is anticipated, which could link the system to other Spanish NHS data sources and enable patients to enter quality of life data [23].

Principles for a national standardised data set in the UK

With one of the largest single-payer health care systems in the world providing cradle to grave care, the UK has unparalleled data potential. The ABPI has considered challenges with the UK's current data capabilities and examples from other countries to produce eight key principles that can help towards realisation of this potential.

Table 1: Key Principles for data collection to support outcome-based payments

Key Principles	
Robust data governance	Robust data governance is essential to maintain compliance with legal, privacy and security controls on data usage in the UK. It also critical to help reassure patients and gain public trust, to avoid large numbers from choosing to opt out of national NHS data collection. This is aligned with Chapter 1 of the DHSC's data strategy [2].
Avoid duplication	It is imperative to avoid duplication of data collection to minimise the administrative burden on clinicians, reduce impact on patients and ensure most efficient use of resources. The implementation of a single national data set that is accessible to relevant stakeholders can avoid duplication by collaborating with existing UK data collections where possible, and only collecting new data when absolutely necessary, to support a range of activities (e.g. pharmacovigilance, outcomes-based payments).
Completeness & quality of data	Minimum viable data requirements should be clearly defined to align with the aim of the registry and ensure completeness and quality of data. These requirements should include aspects that are broadly applicable across disease areas, in addition to best practice approach for determining disease or product specific requirements. The consideration of "*what"* data should be collected for each disease area will require expert agreement on the outcomes of value. All stakeholders, including patient advocacy groups, should have the opportunity to provide input. For a single data system, there are multiple possible used of the data and this will also need to be considered.
Timely access	To avoid duplication and maximise the potential of the data, stakeholders (e.g. Clinicians, hospital pharmacists, MHRA, NHS Digital, NHS England, NICE and industry) should be granted timely access to relevant data. Stakeholder's should only have access to data that is relevant for their specific activities and access should be in alignment with data sharing policies. This is essential for transparency and will enable consistency in the data used for in submissions and appraisals. However, stakeholders should only have access to data that is agreed to be relevant for them and comply with existing policies on data sharing.
National funding	Many disease registries in the UK are run by charities and are unlikely to have access to the required resources to upgrade to generate data that is appropriate for a range of applications (e.g. supporting research, measuring outcomes and monitoring safety). The creation of a single national data set would require a sizeable upfront investment, which would ideally be funded by government, and should consider opportunities to work with charities. The use of public funds to produce a national data set that is owned and maintained by

	public organisations would be preferable to avoid patient and public doubts on the ownership, use and confidentiality of data. Central funding may also improve long term viability of a national dataset.
UK-wide plan	National collaboration on data collection infrastructure and practices across the Devolved Nations will be important to optimise data potential and align with the UK Rare Diseases Framework [24]. This is particularly important for rare diseases, where patient numbers are small and collaboration across nations will be essential to support research and patient care. Collaboration will also be important to support equitable access. For example, if NICE were to issue a positive recommendation for a medicine to be funded in England with an outcomes-based payment model, this option would ideally also be feasible for Wales.
Learning with international partners	Opportunities to learn from other countries experience with outcomes-based payments should be considered. Furthermore, opportunities to collaborate with international partners on broader data collection should be explored, particularly for ultra-rare diseases where there may only be a few patients in the UK.
Assess impact	An evaluation framework should be developed to assess alignment between the original aims and the actual benefits. Assessments scheduled at appropriate time points can provide ongoing feedback for iterative improvements.

Recommendations

1. **It is imperative senior government decision-makers, NHS, clinicians, academia and industry are aligned on the UKs future data strategy and communicate this with clear articulation of the potential benefits and data governance reassurance to the public.** If a significant proportion of patients opt out of NHS data collection, it will have negative implications on the quality and usability of data. There is opportunity for communications to build on Genomics England public engagement work in rare disease and oncology.
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5. **There is strong potential for Scotland or Wales to provide a proof of concept for establishing a national standardised data set that could be adopted across the UK.** This could take the form of exemplar pilots focused on a specific disease area, which could be identified from the ATMP pipeline.

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