Industry Update

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# Access in all areas? A round up of developments in market access and health technology assessment: part 3

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**In this latest update, we explore some of the key updates in market access over recent months including the UK's voluntary scheme for branded medicines pricing, access and growth (VPAG), the first drugs funded by the Innovative Medicines Fund in the UK and the Direct Access Scheme in France, and, finally, the new Institute for Clinical and Economic Review (ICER) value assessment framework in the USA.**

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In November 2023, the UK government announced the new voluntary scheme for branded medicines pricing, access and growth (VPAG), replacing the previous voluntary branded medicines pricing and access scheme (VPAS) [1]. VPAG will be in place from January 2024 until the end of 2028. The purpose of VPAS/VPAG is to guarantee that the NHS branded medicines bill grows by no more than a certain percentage every year, helping the NHS to plan future spending. Any NHS spending above the agreed percentage will have to be paid back to the government by members of the scheme. VPAS had previously been criticized for being uncompetitive due to an annual medicine spending growth limit of only 2%, lowering manufacturer returns and leading to reductions in pharmaceutical investment in the UK. VPAG aims to better support innovation [1] by allowing for a higher and increasing growth rate (2% in 2024, 3.75% in 2025 and 2026, and 4% in 2027 and 2028) [2]. Despite allowing for higher growth, initial forecasts suggest that repayments of £2.6 billion will be made by pharmaceutical companies in 2024, rising to £3.1 billion in 2028 [3]. Companies choosing not to join VPAG are subject to a statutory scheme and must pay a rebate on the sales revenue of branded medicines to the NHS of 21.9% in 2024, 24.0% in 2025 and 26.8% in 2026 [4]. These rebate rates are higher than many similar mechanisms operated by other countries [5]. To distribute repayments fairly, VPAG differentiates between older and newer medicines. The levy applicable to sales of newer medicines will be set each year as required to maintain the sales growth cap. For older medicines, VPAG applies a levy of 10% to all sales. Older medicines that have seen a price reduction of less than 35% will pay an additional top-up levy of between 1 and 25%, depending on the magnitude of the price reduction (i.e., a total payment of between 11 and 35%).

Additionally, VPAG was introduced as a joint government–industry program with the further aim of improving the UK's competitiveness in life sciences and innovation. VPAG scheme members are expected to contribute an estimated £400 million over the 5 years to UK-wide initiatives including expediting clinical trials in the UK (the majority, £300 million); supporting innovative approaches to HTA (£20 million); and improving UK-based manufacturing (£80 million) [6]. VPAG also has ambitions to improve access to and uptake of new medicines that are clinically and cost effective. Steps to achieving this include: an end-to-end pathway guide by the end of 2024 outlining routes to market and how regulatory, HTA and commercial pathways align; the set-up of a UK-wide cross-government working group with industry representation to ensure HTA and payer processes remain interconnected with emerging regulatory pathways; support for implementation of NICE recommendations in local NHS systems; and development by NHS England of tools to track medicine uptake [6]. Improving uptake of

new medicines was a goal of the previous VPAS and therefore it remains to be seen whether these measures will be more effective.

What VPAG does highlight is how this is an additional profit control tool used by the UK (and similar mechanisms exist in other countries) on top of the need for medicines to meet cost-effectiveness thresholds set by NICE. While the UK government positions its changes as a step toward a more investment-friendly pharmaceutical ecosystem, it is unclear if VPAG will be enough to attract further pharmaceutical investment.

Also in terms of UK news was the announcement of the first drugs entering the Innovative Medicines Fund (IMF). The IMF was announced in July 2021 by NHS England to provide faster access to promising new non-cancer medicines while further data are collected, similar to the existing Cancer Drugs Fund (CDF). The CDF was established in 2010 as a dedicated fund to pay for cancer drugs not approved by NICE for routine NHS funding. Its budget grew rapidly to £340 million by 2015. In response to criticisms over funding drugs with limited proven benefits, the CDF was reformed in 2016. It transitioned to a managed access scheme, focusing on gathering more comprehensive clinical and cost-effectiveness evidence. This approach aimed to reduce uncertainties by collecting additional real-world data and more mature trial results. In June 2022, the final IMF principles were published. The fund is allocated an annual budget of £340 million, matching the budget of the existing CDF. While the IMF principles state that its primary function is as a managed access fund to resolve evidential uncertainty for medicines with significant clinical promise, it may also be used as interim funding for recommended medicines prior to routine commissioning to allow earlier patient access [7]. This is likely to have been the case for the three medicines now covered by the IMF, namely, secukinumab (Cosentyx) for treating hidradenitis suppurativa [8], sebelipase alfa (Kanuma) for treating Wolman disease [9], and bulevirtide (Hepcludex) for treating chronic hepatitis D [10]. There has been limited use of the IMF to date, perhaps due to the obligation for companies to cover treatment costs indefinitely for patients receiving treatment via the IMF if the medicine is not recommended by NICE for routine commissioning post the managed access period (these costs could be considerable for patients requiring lifelong treatment). It remains to be seen whether the IMF will predominantly be used for interim funding or as a managed access scheme to address evidential uncertainties.

Also aiming to grant earlier patient access to medicines is the Direct Access Scheme in France, where the first drug entering the scheme was announced in December 2023 [11]. This scheme gives immediate access to drugs following the publication of the service médical rendu (SMR) and l'amélioration du service médical rendu (ASMR) ratings by the Transparency Commission of Haute Autorité de Santé (HAS), and aims to speed up patient access to medicines, which is a notable issue in France. The approved medicine was the gene therapy, etranacogene dezaparvovec (Hemgenix) for the treatment of Haemophilia B [11,12]. The Direct Access Scheme allows medicines with marketing authorization but without early access through existing programmes to be covered by health insurance for 1 year [13,14]. To be eligible, new medicines must have a SMR rating of at least 'major' or 'important', and an ASMR rating of at least 'minor' (level I–IV) [13,14]. Manufacturers will be able to set their own prices for this year (unless the product is already marketed in France for other indications) and the product will be fully reimbursed. At the end of the 1-year direct access period, long-term pricing and reimbursement terms will be determined. To deter manufacturers from setting excessively high prices, after the free pricing period ends and a price is negotiated with the Comité Economique des Produits de Santé, rebates may be claimed from the manufacturer if the negotiated price is much less than that charged during the free pricing period (conversely if the price negotiated is higher, the manufacturer may be eligible to receive additional money from the government). The direct access scheme is broader than the existing early access scheme in France, the Autorisation d'Accès Précoce (AAP), which was established for severe or rare disease populations with a high unmet need (i.e., no authorized therapeutic alternatives). Indeed, Hemgenix was not authorized for the AAP as HAS reasoned that there was already a suitable treatment available, therefore showing how the Direct Access Scheme provides another route for early patient access. Following a 2-year pilot period, the scheme will be reviewed ahead of its possible permanent incorporation in the French reimbursement system [13,14].

In the USA, the Institute for Clinical and Economic Review (ICER) published its updated Value Assessment Framework in September 2023, outlining its approach to future assessments [15]. Key updates include evaluating clinical trial demographic diversity (race/ethnicity, age and sex) to promote discussions around equity and requesting manufacturers explain patient involvement in trial design including their input on outcomes used [16]. While the former may not have a direct impact on how payers view the clinical effectiveness of new therapies, the latter may do, especially when potentially justifying trial end points used. Given it has been several years since the publication of the ISPOR value flower [17], there was a hope that ICER would consider additional broader elements

of value when assessing medicines. While there is some further recognition of this, for example ICER will now always consider productivity in its societal perspective, using an indirect approach to incorporate the impact of an intervention on patient productivity and carer time when data are not available, there is little else that will be quantitatively incorporated. ICER state there is too much methodological uncertainty to quantify elements such as the 'value of hope', health gains in those with more severe illness and scientific spillover effects [15]. The payer perspective will remain the base case, and some elements such as equity, caregiver burden and unmet need will be qualitatively assessed. ICER will however begin evaluating the Generalized Risk-Adjusted Cost-Effectiveness (GRACE) framework as a method to assess health gains relative to severity of disease [16]. Additionally, as the Inflation Reduction Act has brought more attention to medicine price decreases later in their lifecycle, ICER is considering methods to perform cost–effectiveness analyses accounting for this [16]. ICER's evidence reports are gaining increasing traction with US payers and decision makers, and therefore it will be important for manufacturers to plan to generate the evidence that ICER will assess. While ICER's updated value assessment framework represents a move toward equity and societal impact considerations in drug evaluations, there is an argument to be made that more could have been done as undervaluing medicines may ultimately limit patient access to them and reduce the incentives for future drug development.

Finally, in December 2023, the Canadian government made a surprise announcement regarding the establishment of the Canadian Drug Agency (CDA), building on but replacing the work of the existing Canadian Agency for Drugs and Technologies in Health (CADTH) [18]. New priorities of the CDA will include improving appropriate use of medications to improve patient health and increasing real-world data collection across Canada to improve decision making [18]. These changes may reflect the intentions of the Canadian Government to move from health technology assessment to health technology management (i.e., not just performing HTA at a single point in time but having reassessments) [19]. The Canadian government has announced it will invest an additional CAD$89.5 million over 5 years to establish the CDA [18].

Overall, these recent updates demonstrate a global aim to improve access to medicines and promote innovation. Yet, the success of these initiatives in achieving their intended outcomes is not guaranteed. As these changes are implemented, their true impact on the pharmaceutical landscape will become clearer.

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