Windrose Consulting Group Global News Roundup: Q2 2023


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  - Spain
  - Sweden
  - Ireland
  - Mexico, Colombia and Cuba
  - Japan
  - China

Kristina Funk

Proposed reform of EU pharmaceutical legislation could change the innovation landscape in Europe

DETAILS

- On April 26th, 2023, the European Commission proposed a reform to the EU's pharmaceutical industry, which aims to increase availability, accessibility, and affordability of medicines
- Key potential changes include*:
  - Reduction of regulatory protection / exclusivity from 10 to 8 years, unless certain conditions are met (such as launch in all member states within 2 years of marketing authorization)
  - Reduced authorization time due to simplified EMA structure (e.g., reduction to 2 main scientific committees)
  - Transferable data exclusivity vouchers could be granted for investment in novel antimicrobials; these vouchers increase data protection by 12 months

INDUSTRY IMPLICATIONS

- The proposed reform could greatly affect market access and pricing in Europe, and industry groups and executives have already voiced concerns
- With the earlier loss of exclusivity (10 to 8 years), there will likely be a significant reduction of revenue and profit opportunity for many drugs
- Extensions will be granted for launching drugs in all EU member states within 2 years of marketing authorization, address unmet medical needs, or have comparative clinical trials
- However, launching in all member states within that set timeframe may not be feasible and could represent an unrealistic target for companies
- For orphan drugs, there would be more challenging conditions for orphan designation, and the standard orphan market exclusivity reduced from 10 to 9 years which may decrease the attractiveness for EU orphan drug investment**
- If the legislation is passed, the pan-EU HTA may become increasingly important for a successful European launch

ADDITIONAL RESOURCES

EUROPEAN COMMISSION
PUBLIC HEALTH

*Additionally: Increased obligations for companies to avoid drug shortages (e.g., quick report of shortage, shortage prevention plans) and information on direct financial support for R&D from public bodies needs to be disclosed online, accessible for the public. **Similarly, extensions can be applied, for example for launch across member states (1 year)*

James O'Brien

France launches a two-year “direct access” scheme experiment to accelerate and improve patient access to innovative medicines

DETAILS

- In May 2023, France has begun a two-year trial of a new “direct access” scheme, where “innovative” drugs can achieve access immediately after TC assessment with initial free pricing, without the need for a CEPS price negotiation
- Drugs are eligible if they are classified as “innovative”, have SMR Important, ASMR IV or better, are not covered by early access or compassionate use programs, and are not already launched in other indications in FR
- The free pricing period lasts for one year, after which the CEPS-negotiated pricing applies, and retrospective rebates may be implemented if the free price is higher than the negotiated price
- If the trial is successful, “direct access” will become a permanent feature of FR market access from 2025 onwards

INDUSTRY IMPLICATIONS

- The definition of “innovative” therapy has been left purposefully vague for now, so it remains to be seen which drugs will be eligible for this fast-track route
- As with DE, the free-pricing period provided by the “direct access” scheme represents a launch sequencing opportunity, whereby launching first in FR could increase prices in countries that reference FR in International Reference Price baskets
  - The introduction of a one-year free-pricing period in FR (for eligible drugs) comes at a time when the DE free-pricing period has been reduced from one-year to 6 months
- Nonetheless, the advantage of this period is limited by the prospect of retrospective rebates if the free price is significantly higher than the negotiated price
- The “direct access” scheme represents a move by France to encourage the development and access of innovative drugs
  - This reform comes in contrast to many other European countries, such as Germany, who are restricting pricing and access incentives in order to manage post-covid health budget deficits

ADDITIONAL RESOURCES

FRENCH MINISTRY OF HEALTH

HEALTHCARE INNOVATION PLAN

Steven Lin

The FDA approves first two vaccines for respiratory syncytial virus (RSV) for individuals 60 years and older

DETAILS

- In May 2023, the FDA approved two vaccine candidates, GSK's Arexvy and Pfizer's Abrysvo, for adults aged 60 and older, making them the industry's first vaccine offerings for RSV
  - Respiratory syncytial virus is a respiratory virus that usually causes mild, cold-like symptoms; RSV is a common cause of lower respiratory tract disease (LSTD), which may lead to life-threatening pneumonia and bronchiolitis
- In June, the Advisory Committee on Immunization Practices (ACIP) recommended the RSV vaccines for the FDA-approved population to prevent LSTD, based on shared clinical decision-making between patients and healthcare providers (HCPs)
  - In addition, the vote by ACIP experts was 9-5 to recommend the vaccine for adults 65+, compared to 13-0 for 60+ (one abstention)

INDUSTRY IMPLICATIONS

- The approval of the first RSV vaccines and the recommendation from the ACIP represent a critical milestone in addressing the high unmet need for RSV, especially in higher-risk population
- However, the ACIP is hesitant to endorse the vaccines for the 65+ population by noting that individuals "may" receive a single dose of the vaccine under consultation with their HCPs, citing concerns about the lack of enrollment for people of advanced age in the clinical trials
- In the absence of a strong endorsement, both companies preparing to launch this fall could face significant sales challenges as the actual uptake may be uncertain, especially considering the potentially high costs of the vaccines ($180-$295)
- Aside from adults, future RSV indications such as for infants and younger individuals of high risk could further evolve the competitive landscape, as additional FDA decisions regarding the use of RSV vaccines become available in August 2023

ADDITIONAL RESOURCES

FDA

CDC

Itamar Carrillo

Ministry of Health to Potentially Implement New Criteria for Drug Price and Reimbursement

DETAILS

- In June 2022, CAPF published their recommendations for drug pricing and reimbursement decisions in Spain which would be made using the following priority criteria:
    1. Relevant additional clinical benefit: key driver for price setting, this would require “good quality” evidence[1] and include patient’s perspective[2]
    2. Efficiency: cost-effectiveness and cost-utility are likely to be evaluated in relation to health and quality of life gains, and a budget impact to guarantee sustainability for the healthcare system
    3. Uncertainty[3]: must be explicitly considered in the budget, cost and efficacy assessments; if it is high, the manufacturer must determine the conditions that must be met for its management
- These recommendations aim to enhance the pricing and reimbursement process; however, their formal inclusion on the Spanish legislation is still to be determined

INDUSTRY IMPLICATIONS

- As the key consideration is the additional clinical benefit rating, it is likely pricing may be based heavily on the drug’s assigned category (i.e., clinical benefit, additional clinical benefit, relevant additional clinical benefit or therapeutic alternative of similar clinical benefit)
    - Therefore, it will be crucial for manufacturers to understand the criteria for each category, and keep track of which analogues are successful in achieving different categories
- While the efficiency aspect will be important, it is unlikely to be the final driver for decisions, and therefore there may be some flexibility
- While uncertainty has always played a role in decision-making, formalizing it as part of the assessment may provide opportunity for leverage in pricing & reimbursement to secure lower prices
- Nonetheless, there may be greater opportunity to leverage contracting to mitigate uncertainty i.e., coverage with evidence development, special payment options and close monitoring of drug use

ADDITIONAL RESOURCES

MINISTRY OF SANIDAD

[1] Head-to-head trials vs adequate comparator, if this is not possible, other studies will be accepted if clinical uncertainty is contained; [2] patient perspective variables include: survival, QoL, clinical variables;
[3] uncertainty can fall into any of the following categories: safety, efficacy, additional clinical benefit, QoL, budgetary impact, its use in real life; CAPF = Advisory Committee Pharmaceutical Provision of the National Health System

Adrian Jung

ICER/NICE/CADTH Collaborates with International Partner Agencies to Streamline the Confidential Marking Process

DETAILS

- Previously, CADTH, ICER, and NICE have accepted unpublished data, as they have been asked to make decisions earlier in the lifecycle of health technologies, and have redacted it from public documents
- However, to streamline processes and increase transparency, CADTH, ICER, and NICE have aligned on a new approach to handling clinical data
- For evaluations starting after April 2023, NICE technology appraisals and CADTH assessments will no longer routinely redact clinical data that has not yet been published, and ICER will only allow redaction of data that is formally planned to be published within the next year

INDUSTRY IMPLICATIONS

- Greater corroboration amongst HTA agencies may streamline reviews and increase acceptance of global evidence
- For CADTH, the goal is to maintain existing global publication plans and/or timelines for application filing
- In the UK, these changes were a part of NICE's new "proportionate approach" to evaluations of health technologies, which will apply streamlined and faster evaluations to simpler, low-risk treatments
- As part of a pilot project that began last year, NICE has so far recommended 5 treatments, and made the technology appraisals process up to 45% faster

ADDITIONAL RESOURCES

CADTH

NICE

ICER

CADTH = Canada's Drug and Health Technology Agency, HTA = health technology assessment, ICER = Institute for Clinical and Economic Review (US), NICE = National Institute for Health and Care Excellence (UK).

Amy Morgan

From January 2024, a new drug can apply for NHIA listing at the same time as regulatory approval submission

DETAILS

- In Taiwan, all new drugs are evaluated and get approved by the Taiwan Food and Drug Administration (TFDA); subsequently, the reimbursement of a new drug is decided by the National Health Insurance Administration (NHIA)
- The current reimbursement lag in Taiwan is longer than 1 year; the reasons for the lag differ between drug categories and include:
  - High budget impact of certain therapies
  - Determination of the reimbursement price
  - Pharmaceutical price-volume agreements
  - New payment models for exceptionally high-cost, one-off curative therapies
- A study conducted by the Institute of Health and Welfare Policy, National Yang Ming University, recommended that the government should re-examine the current function of the regulatory and reimbursement systems to improve patient access to innovative therapies

INDUSTRY IMPLICATIONS

- Allowing new drugs to apply for NHIA listing in parallel to regulatory approval submission, could potentially speed up access by 2-3 months
- While the new process will be applicable to all drugs, it will not mitigate the need for price negotiation

ADDITIONAL RESOURCES

FDA TAIWAN

Amy Morgan

Lif*, the NT Council and the regions' cooperation model for medicines propose measures to facilitate access to combination treatments

DETAILS

- Combination therapies have an increasing role in the standard of care of various oncology indications
- However, combinations face major challenges during payer assessments, as their per-patient cost is considerably higher than for monotherapies, making it difficult to meet cost-effectiveness criteria
- The challenge has been especially clear in oncology and haematology, where the NT Council have had several urgent combinations that could not be approved for use

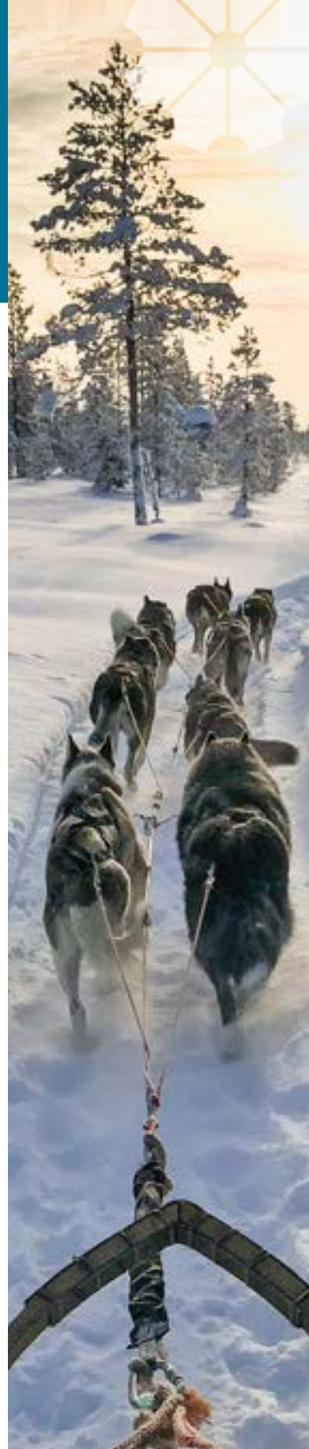
INDUSTRY IMPLICATIONS

- National based agreements, not unlike those already in place for PDL-(1)s, are envisioned where the price of combination therapies will be subject to health economic evaluations using ‘weighted’ prices combined with ‘willingness to pay’ to form the basis of a national agreement
- The NT Council would determine the weighting by evaluation across applications
- Manufacturers would then be invited to provide tender bids - after each round, it would be determined whether the price level resulting from the bids allows for a positive recommendation
- The new approach is anticipated to be reserved for a limited number of combinations that are difficult to price using conventional methods

ADDITIONAL RESOURCES

LIF

**Läkemedelsindustriföreningen (Lif) is the trade association for the research-based pharmaceutical industry in Sweden; NT = new therapies council*



Amy Morgan

The reimbursement of rare disease medicines in Ireland is still lagging behind EU counterparts

DETAILS

- Ireland is currently lagging behind Romania, Hungary and other Eastern European countries in the provision of orphan drug
- The Irish system does not include an orphan drug policy which contributes towards the low availability of orphan drugs in Ireland:
  - Presently, the same HTA, is carried out for new medicines irrespective of type
  - This includes a QALY assessment with the threshold set at €45,000 per QALY
  - The quantitative data is often limited by sparse clinical or epidemiology data around a rare disease; as such, a QALY-based HTA will result in huge uncertainty
- While the allocation of dedicated funding for new medicines has had a positive impact on the reimbursement over the past 2 years, more funding is required
- A new Bill* is seeking to establish a separate process for assessing orphan products, allowing them to be streamlined and assessed on their individual merit
  - The Bill is currently still held up in the legislative process

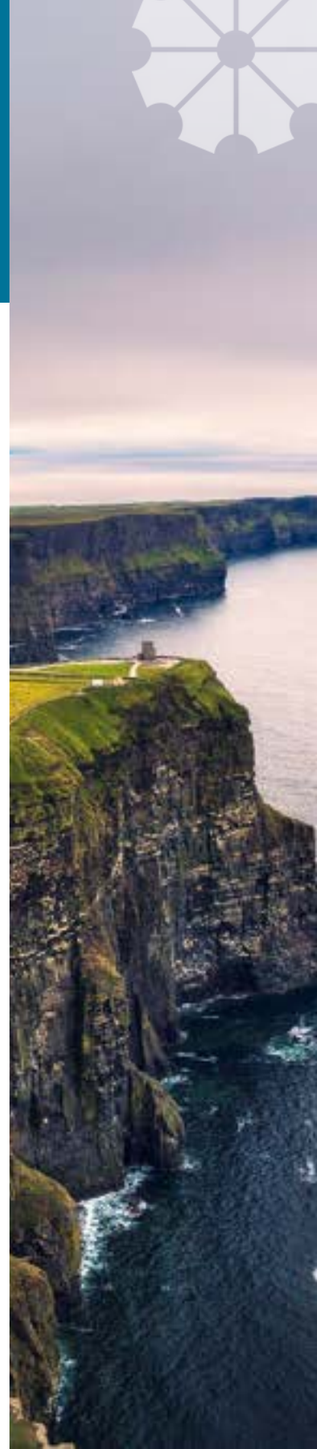
INDUSTRY IMPLICATIONS

- The new bill proposes that no ICER will be considered relevant to the assessment of orphan drugs
- Moreover, the Health Service Executive (HSE) will be required to consider the level of certainty provided via risk sharing commercial agreements with manufacturers
- If successful, this would increase feasibility for manufacturers seeking to launch orphan drugs in Ireland

ADDITIONAL RESOURCES

FIANNA FÁIL TD

** Orphan Drugs And The Health (Pricing And Supply Of Medical Goods) (Amendment) Bill 2021*



Amy Morgan

Mexico, Colombia and Cuba, are moving ahead with plans to create a new Latin American and Caribbean Medicines Agency

DETAILS

- The aim of the Latin American and Caribbean Medicines Agency will be to help the region become more self-sufficient:
  - To achieve regulatory convergence and mutual recognition of authorizations to guarantee effective access to quality, safe and effective medicines for the region
  - Stimulate and support the R&D of innovative products and provide regulatory certainty throughout the region
  - Support local production and integration of local supply chains
  - Explore public procurement mechanisms to guarantee access and sustainable healthcare financing
  - Prioritize “self-sufficiency,” such as contracts with regional manufacturers
  - To increases preparedness in future emergency situations given the lessons emerging from the recent pandemic

INDUSTRY IMPLICATIONS

- The pharmaceutical industry is generally in support of a new regional regulator that follows in the model of the European Medicines Agency; yet there are some reservations
- While the governments of several other countries have voiced support for a Latin American Medicines Agency, including Bolivia, Dominica, Ecuador, El Salvador, Honduras, and Jamaica; Brazil and Argentina have so far rejected the idea of joining the initiative
- The risk to companies is that they would have to navigate additional layers of regulation to comply with both national and regional regulations
- Such multi-layered compliance requirements would mean added costs, time and resources for companies, including hiring new staff to manage the new regulatory processes

ADDITIONAL RESOURCES

LATIN AMERICA NEWS

Amy Morgan

Japanese, American and European pharma associations have issued recommendations for strengthening Japan's 2024 Drug Pricing Reforms

DETAILS

- Reform is needed to restore sustainable growth to the market and ensure that Japan is not left behind in developing and accessing the world's latest treatments and vaccines
- Three policy proposals have been put forward for consideration by the Ministry of Health, Labour and Welfare to achieve a rapid and stable supply of pharmaceuticals for the 2024 National Health Insurance (NHI) Drug Pricing Reform:
  - Proposal 1: Maintain Drug Prices During the Patent Period - most innovative medicines in Japan face commercial uncertainty with frequent price cuts, in contrast to the practice of other leading countries
  - Proposal 2: Improve Initial NHI Price-Setting - the current methods used to set the initial NHI prices of new medicines have restrictive criteria that do not reflect the value of innovation
  - Proposal 3: Reduce Inefficient Spending - increase the clinically appropriate uptake of generics and biosimilars; and promote prompt price reductions for long-listed products

INDUSTRY IMPLICATIONS

- Improving price maintenance:
  - It is proposed to exclude innovative medicines from the market price-based revisions during the patent period, meaning that approximately half of innovative medicines today would be saved from annual price cuts
  - In addition, improvements to the expansion and spillover repricing rules have been suggested which would protect innovative products from the price reductions when these rules are triggered
- Improving NHI price setting:
  - Two-thirds of innovative medicines fail to receive a price premium at NHI listing, and if they do, they only receive the minimum amount
- Wider assessment criteria are being proposed to allow for a more holistic evaluation of product value as well as expanding the scope of comparators allowed

ADDITIONAL RESOURCES

JPMA

Amy Morgan

The Ministry of Health, Labor and Welfare (MHLW) publishes annual repricing, affecting 2/3 of the listed drugs

DETAILS

- The scheduled biannual price adjustments that have historically been used in Japan as a cost-reduction method have been adjusted in recent years, with 2023 marking one of these off-year price revisions and the fifth year in a row that drug prices have reduced
- National health insurance-listed products with more than a 4.375% rate of price discrepancy between their NHI prices, and their market prices experienced an off-year price reduction or price increase

INDUSTRY IMPLICATIONS

- Off-year price adjustments have added to the complexity of the Japan pricing system, possibly reducing the attractiveness of the Japanese market for future launches by global manufacturers
- Off-year price adjustments could impact the commercial potential of current and pipeline products; therefore, it will be critical for manufacturers to evaluate their long-term pricing strategy in order to engage in accurate forecasting

ADDITIONAL RESOURCES

PHRMA JP

NAVLIN DAILY

JPMA

JAPAN DRAFT BUDGET

Amy Morgan

China's Administrative Measures for Supervising Online Sales of Drugs allows the online sales of prescription drugs nationwide after regional pilot programs in Hainan and Shenzhen

DETAILS

- Marketing authorization holders are now able to sell their prescription drugs online nationwide
  - The following are not to be sold online: vaccines, blood products, anaesthetics, psychoactive drugs, toxic drugs for medical use, radioactive drugs, and chemical precursors
- Nonetheless, they must comply with strict rules to avoid sales based on fake prescriptions and the repeated use of one prescription by multiple purchasers
  - E-commerce platform companies, also known as third-party online sale platform companies, will be required to ensure that the obligations of these new measures are met

INDUSTRY IMPLICATIONS

- The policy could lead to the emergence of a market worth more than RMB 1 trillion ($161 billion) as sales shift from hospitals to online pharmacies
- The policy will also help reform the Chinese market that has been solely controlled by state hospitals and distributors
  - Offline distributors such as Shanghai Pharmaceuticals and Chinese Medical Systems Holdings may be threatened as manufacturers sell directly to hospitals and businesses without going through a middleman
- Third-party online sales platform companies could also support the online pharmacy sector develop omnichannel service competence including diagnosis, treatment, drug delivery and health/disease management
- For pharmaceutical companies, it is predicted that the future of doing business in China could involve a whole new set of partners

ADDITIONAL RESOURCES

NMPA

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