NEWS DIGEST

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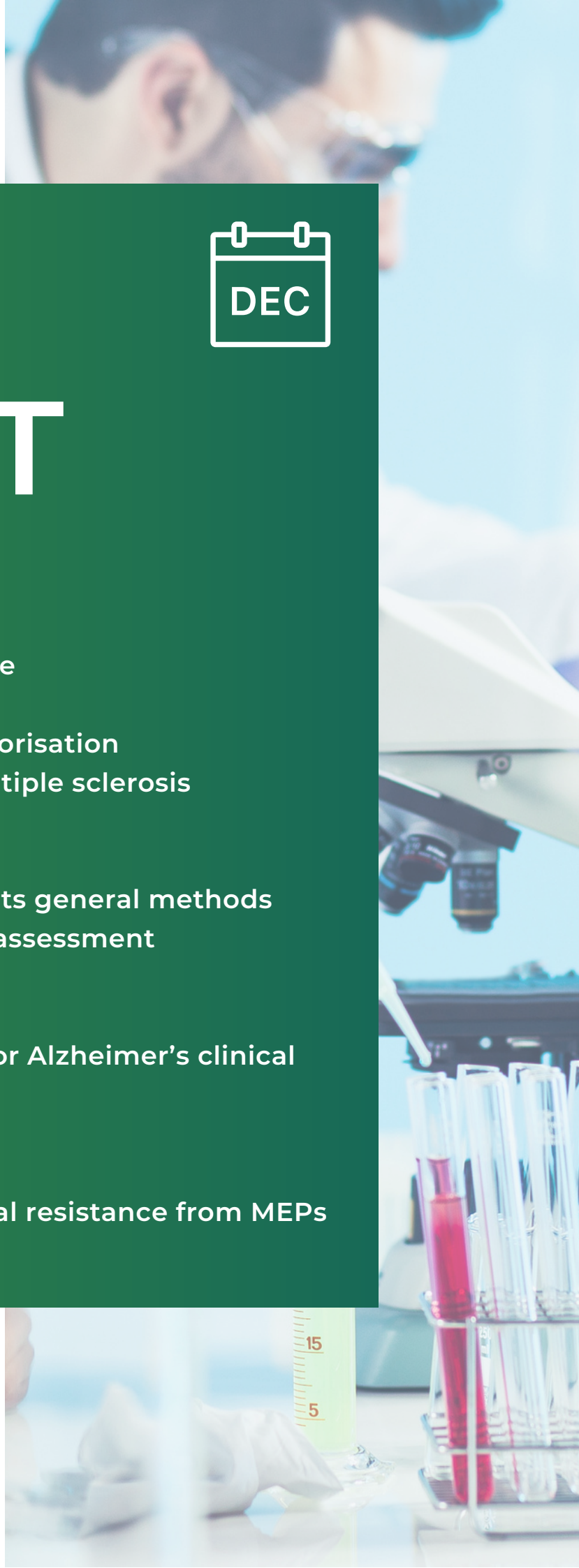

Remap

UK: NICE releases draft guidance recommending three treatments for COVID-19

The National Institute for Health and Care Excellence (NICE) has published draft guidance recommending the use of three COVID-19 treatments.

Two recommended treatments are indicated for use in a hospitalised setting in patients requiring supplemental oxygen: Roche's RoActemra (tocilizumab) and Eli Lilly's Olumiant (baricitinib). Pfizer's Paxlovid (tirmatrelvir plus ritonavir) is recommended for use in non-hospitalised settings.

NICE advised against five further treatments.

Casirivimab plus imdevimab and sotrovimab, as well as Tixagevimab plus cilgavimab are not recommended due to uncertainty whether these treatments were effective against the Omicron variant.
Molnupiravir and remdesivir are not recommended as, while there is clinical evidence that they are effective at treating COVID-19, they were not found to be cost-effective.

Helen Knight, interim director of medicines evaluation at NICE, declared: "We are ready to work with all the companies whose products have not been recommended in this draft guidance, and with NHS England, to explore whether there is scope for any commercial or other solutions that could address the issues highlighted by the independent appraisal committee".

Moreover, the evaluation for tixagevimab plus cilgavimab as a COVID-19 prophylaxis (preventative treatment) is being evaluated separately from the COVID-19 treatments. Final guidance for this evaluation is anticipated to be published in the spring of 2023.

Remap

UK – NICE releases guidance recommending targeted treatment for a rare form of aggressive lung cancer

The National Institute for Health and Care Excellence (NICE) has published final draft guidance recommending Takeda's Exkivity (mobocertinib) for the treatment of the epidermal growth factor receptor (EGFR) exon 20 insertion gene mutation positive form of advanced non-small-cell lung cancer (NSCLC).

NSCLC is the most common form of lung cancer, accounting for 85% of the 1.8 million newly reported cases worldwide. This specific mutation is rare and aggressive, making up 2% of patients with NSCLC, and currently has no standard treatment, identifying a large unmet need for treatment. Mobocertinib, taken as four capsules per day at home, functions by specifically targeting the mutation to slow cancer cell growth, and positive guidance was conferred in light of the positive results of a phase ½ trial of Exkivity evaluating its safety and efficacy.

Moreover, indirect comparisons with real-world evidence on immunotherapies and docetaxel with or without nintedanib suggest that mobocertinib increases life expectancy and overall survival.
Helen Knight, interim director of medicines evaluation at NICE, said: "This is the first treatment approved by NICE that targets this specific gene mutation in people with advanced non-small-cell lung cancer. The evidence shows it not only extends people's lives but also extends how long people have before their cancer gets worse".

The treatment was licensed through Project Orbis, an international programme to review and approve promising cancer drugs helping people access treatments faster, and the company has a confidential commercial arrangement through a patient access scheme, making mobocertinib available to the NHS with a discount.

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UK – Otsuka receives vital Lupkynis MHRA authorisation for the treatment of Lupus Nephritis

Otsuka has announced that the MHRA (Medicines and Healthcare Products Regulatory Agency) has authorised Lupkynis (voclosporin) for use in combination with mycophenolate mofetil for the treatment of active class III, IV, or V Lupus Nephritis in adults in England, Scotland, and Wales. Voclosporin is the first and only oral CNI (calcineurin-inhibitor immunosuppressant) licensed in Great Britain for the treatment of active Lupus Nephritis in adults.

Lupus Nephritis is a severe manifestation of systemic lupus erythematosus (SLE), a chronic autoimmune disease that can lead to irreversible kidney damage. Around 40-60% of individuals with SLE develop Lupus Nephritis, and data from studies conducted in 2012 showed 60,000 sufferers of SLE in the UK.

The CNI Voclosporin inhibits calcineurin in a dose dependant manner, and its immunosuppressant activity inhibits lymphocyte proliferation, T-cell cytokine production, and the expression of T-cell activation surface antigens.

Authorisation from the MHRA is a result of positive findings from the pivotal phase 3 AURORA-1 study and comparative data from the AURORA-2 study in which the effect of a voclosporin, mycophenolate mofetil (MMF), and corticosteroid combination was compared to just MMF and corticosteroids.
Ryan Gynne, managing director of Otuska, stated: “The MHRA’s authorisation of Lupkynis represents a significant development for Lupus Nephritis patients in Britain, offering voclosporin as a new combination treatment option for eligible patients”.

UK – World-first clinical trials for stem cell transplants that may be used as first-line treatment for aggressive multiple sclerosis begin in the UK

A world-first trial has been launched in the UK that could allow for stem cell transplants to be given as first-line treatment to patients with aggressive multiple sclerosis (MS).

The £2.3 million StarMS study, which has launched in Sheffield and is steadily launching across the UK, will be the first study to compare the efficacy for autologous haematopoietic stem cell transplantation (AHSCT) head-to-head with four other treatments that have been effective in clinical trials (alemtuzumab, ocrelizumab, ofatumumab and cladribine).

The trial will build on the results of the MIST trial, which was the first in the world to show the effectiveness of stem cell transplantation at reversing disability in sufferers of MS, and that AHSCT was more effective than the disease-modifying drugs available in reducing the risk of disability accumulation in patients with aggressive MS.

MS is a disabling disease in which impairment to the nervous system occurs as the immune system attacks the protective myelin sheath covering nerves – affecting 100,000 in the UK and 2.3 million globally. AHSCT is an intensive treatment that rebuilds a patient's immune systems using stem cells derived from their own blood and bone marrow that essentially "resets" the immune system to a condition prior to the effects of MS.

Professor John Snowden, chief investigator and consultant haematologist at Sheffield Teaching Hospitals NHS Foundation trust, stated: "AHSCT has been shown to be highly effective in stabilising, and even reversing disability, in certain patients with MS". He further said: "The trial will measure how good and safe AHSCT is when compared head-to-head with the latest leading treatments for multiple sclerosis. In this way, we hope to determine the exact place of AHSCT in the modern treatment pathways for patients with severe MS".

The new StarMS trial is being funded by a partnership between the Medical Research Council and National Institute for Health and Care Research (NICE)

Germany – IQWiG proposes changes to its general methods with a focus on cost benefit assessment

IQWiG has created a new draft (7.0) of its general methods. The general methods describe, transparently, how they prepare their reports. The primary change is the chapter 4 which focuses on the cost-benefit assessment of medicinal interventions. The public have until the 20th January 2023 to comment on the additions and updates included in draft 7.0 of the general methods of IQWiG.

Proposed changes

Since AMNOG came into force in 2011, chapter 4 of the general methods has been used to provide guidance in the pricing of new drugs when the early benefit assessment has been completed and the price negotiations have failed. The change in procedure aims to setting the maximum price in accordance with an earlier legal norm. IQWiG head Jürgen Windeler explains the reason for changing the current rules "In view of the ever-increasing prices for new patent-protected drugs, the institute now wants to be able to process orders for a cost-benefit assessment in a reasonable amount of time. Even if there are currently no signals for such an order were sent by those involved in the healthcare sector, an adjustment of our methods therefore seems appropriate.” In the course of the fundamental revision of chapter 4 on the cost-benefit assessment, section 1.4 on health economics was also completely rewritten.

IQWiG has newly included sections 2.1.3.2, 2.1.3.3 and 3.3.4 in its methods paper. In it, the Institute describes the methodical procedure when it is commissioned by the G-BA to create an AbD concept (AbD = application-accompanying data collection).

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Currently the G-BA have been able to request an AbD including an evaluation from the manufacturer for drugs used to treat rare diseases (orphan drugs) in order to generate additional data for quantifying the additional benefit. IQWiG then provides the necessary methodological support for this. In the revision the "AbD concept" and "Search for indication registers" are regulated like the AMNOG dossier assessments for the purpose of the early benefit assessment of new drugs in § 35a of the Social Security Code V (SGB V) . IQWiG now clearly presents this expanded product range for assessments in accordance with § 35a SGB V in Chapter 2 of its General Methods Version 7.0.

IQWiG has fundamentally revised its understanding of evidence-based medicine (EBM) in section 1.3 of the methods paper. Section 3.5 on diagnostic procedures has also been revised and expanded.

There are also major changes in Section 3.2.1. Here, the methodological approach to dealing with missing data or a possible “reporting bias’ was significantly streamlined and the heavily formalized approach was replaced by a situation-related approach. Moreover, the details for how they use statistical significance tests and confidence intervals to support decision-making has been refined.

How to comment on the proposed changes

Written comments on the new content can be submitted until January 20, 2023. The Institute will review all statements, discuss them if necessary and acknowledge them accordingly. The relevant sections will then be revised if necessary and version 7.0 of the methods paper will be published on the Institute's website.

FR – Launch of national registry for Alzheimer's clinical trials and studies

The Vaincre Alzheimer Foundation is launching a unique national directory listing current and future clinical studies on Alzheimer's and related disease carried out by industry and academic sponsors in France.

A study conducted by the Foundation in 2017 showed that 87% of French people do not know where, or who to turn to if they want to participate in ongoing clinical trials.

The aim of the registry is to inform patients, healthcare professionals and the general public about developments in clinical research in the field of Alzheimer's and related disease. Further, the Foundation hope it will improve patient recruitment in French clinical trials, and access to innovative therapeutics.

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EU: Call to action on antimicrobial resistance from MEPs

An event was hosted by the European Parliament ahead of World Antimicrobial Awareness Week to discuss antimicrobial resistance (AMR) in the EU and member states, bringing together members of the European Parliament (MEPs), pharmaceutical company Shionogi, the Active Citizenship Network and the MEPs Interest Group on 'European Patients' Rights and Cross-Border Healthcare. The aim of bringing these high-level European policy makers and experts together was to discuss the development of new antibiotics and initiatives for national health technology authorities to fight antimicrobial resistance, and to explore collaborative and governance models to better implement actions and improve best practices to fight AMR.

Antimicrobial resistance has long been seen as a priority health threat by the European Health Union and World Health Organisation, with it branded a 'slow tsunami that threatens to undo a century of medical progress' by the director general at WHO. In 2019, over one million deaths were directly caused by bacterial AMR and five million were partly related to it; by 2050 it is estimated that AMR could lead to as many as 10 million deaths per year by 2050 and cost up to $100 trillion to global economic output. There is currently a reliance on antibiotics in all aspects of modern healthcare, thus without significant change we risk endangering common surgeries and making complex therapies impossible.

The event resulted in MEPs and stakeholders alike calling for urgent action in response to antimicrobial resistance. It reinforces concerns about AMR from the European Health Union and highlights the importance of bringing about policy change ahead of the planned European Council Recommendations on AMR in Q4 2022.

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We always welcome your thoughts and opinions on the topics raised here.

If you'd like to share anything or hear how we can support you in getting your product to market, email Paul and Graham, managing directors, today at: contact@remapconsulting.com.

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