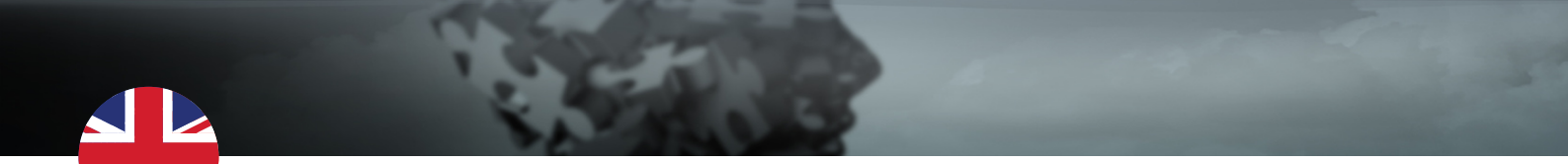
NEWS DIGEST

MAR
15

- NICE issues conditional recommendation for digitally enabled therapies to treat depression and anxiety in adults
- Fimea launches pilot procedure for the evaluation of new hospital-only drugs
- The German Pharmaceutical association insists benefits must continue for Orphan drugs
- Promotion of RWE evidence in the approval process by the German G-BA and the Belgian KCE

Remap



NICE issues conditional recommendation for digitally enabled therapies to treat depression and anxiety in adults

Following a consultation, The National Institute for Health and Care Excellence (NICE), has conditionally recommended eight digitally enabled therapies which utilise cognitive behavioural therapy (CBT) techniques and the support and involvement of an NHS Talking Therapies Clinician. These therapies address depression and a range of anxiety disorders, including PTSD and body dysmorphia.

The conditional recommendation of these therapies are the 6th and 7th early value assessments that have been initiated using the new NICE rapid assessment process for promising medical technology, and will allow for further evidence to be collected on the therapies prior to an official recommendation and use within the NHS
NHS Digital estimates that 1 in 6 people report experiencing a common mental health problem, including depression and anxiety disorders, in any given week. This burden is coupled with the high demand for NHS talking therapies, highlighting the need for promising new technology that may alleviate this burden and add to the range of evidence-based treatments that can be offered.

Mark Chapman, interim director of medical technology and digital evaluation at NICE, stated: “We want these new treatment options to be available for people to use as quickly as possible and we also want to make sure they are clinically effective and represent good value for the NHS. The additional evidence collected during this period will help us do that.”

Remap

Fimea launches pilot procedure for the evaluation of new hospital-only drugs

At the beginning of March Fimea launched a new pilot program with the aim of harmonising the methods of assessment related to the introduction of outpatient and hospital-only medicines. This originates from the reforms of pharmaceutical affairs, instigated by the Ministry of Social Affairs and Health in 2019.

Currently the early stages of an evaluation for outpatient medicines and hospital-only medicines are different and assessed by an application procedure (outpatients) or a model focused on initiatives by officials (hospital-only). In Fimea's regular hospital-only drug assessment, Fimea selects the assessment topics each month from among the CHMP's positive opinions, sends an information request to the company in question and launches the assessment. In the pilot procedure however, the initiative will come from the company. The drug manufacturer informs Fimea of the new hospital-only medicine and the anticipated date for submitting the application, then proposes the scope of the assessment. Fimea will comment on the scope of the assessment and approve or reject the topic as an application procedure pilot. The company will then submit its final application at the time of CHMP's positive opinion and the assessment will then be launched.

If the program is seen to be successful, it will be extended to a full application model, enabling the standardisation of elements such as application requirements and fees. This would eventually increase the coverage, predictability and speed of hospital-only drug evaluations.

The German Pharmaceutical association insists benefits must continue for Orphan drugs

Following the announcement that the European Commission is currently revising the EU drug legislation, which is likely to cause benefit restrictions for orphan drugs. The Federal Association of the Pharmaceutical Industry eV (BPI) insists that there must be no cuts in the incentive system for the development of orphan drugs.

There are currently around 200 medicinal products for around 8,000 known rare diseases. However, the need for research remains high. BPI Managing Director Dr. Kai Joachimsen. “The incentives and the therapies that result from them are glimmers of hope for the patients affected. We must not make any compromises when it comes to finding therapies where adequate treatment options have so far been lacking.”

“Investment decisions are already being made difficult for pharmaceutical companies," says Joachimsen. "The recently passed Statutory Health Insurance Fund Financial Stabilization Act counteracts the idea of promoting orphan drugs: Drugs only receive a special status in the AMNOG if they are below an annual turnover threshold that has been reduced from formerly 50 to 30 million euros. As the BPI, we have repeatedly pointed out that this is accompanied by a step backwards in the care of patients with rare diseases. It is now all the more important that the future EU drug legislation sticks to the proven funding instruments” Continues Joachimsen.

Source: Orphan Drugs: Förderung weiterhin von hoher Relevanz. BPI. https://www.bpi.de/alle-themen/ansicht-themen-nachrichten/orphan-drugs-foerderung-weiterhin-von-hoher-relevanz. Accessed 9th March 2023

Promotion of RWE evidence in the approval process by the German G-BA and the Belgian KCE

Members of IQWiG along with members of KCE, the Belgian HTA agency are promoting the use of RCTs which include real world evidence.

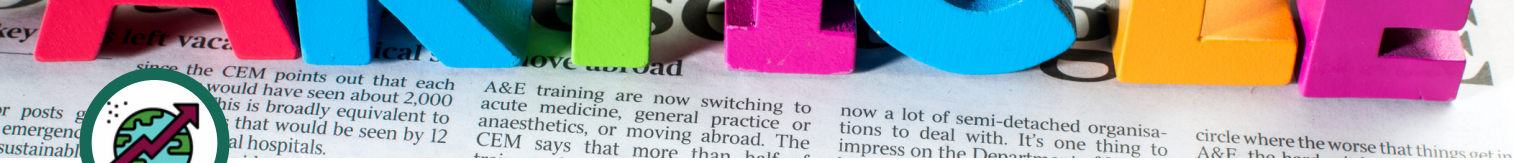
Due to acceleration of drug approvals more drugs are coming to market with a limited clinical study base and Real World Evidence (RWE) is more regularly being obtained post launch. Whilst in the US clinical trial data can include RWE for drug approval the EMA stipulates that the data must not come from clinical trials (though the authors predict this will change). However, as it cannot be used in Europe increasing amounts of data is coming from single arm trials which lack comparison with the standard of care making clinical and reimbursement decisions challenging.

Members of IQWiG and the KCE presented 15 approaches for making RCTs with RWE easier, faster and cheaper to carry out. Some of these approached are highlighted below:

- As patient registers grow and become more standardised it will be easier to recruit patients
- Create standardised patient registers for rare diseases
- Set up a European wide trial infrastructure
- Platform studies that the same control group could be used against multiple active ingredients
- Include those affected in the study design to help to ensure the collection of patient-relevant evidence
- Emphasise the importance of multi-purpose studies which address regulatory approval and benefit assessment to avoid a duplication of work
- Expand inclusion criteria to reflect the target populations

Source: Effiziente RCTs mit Real World Data für Nutzenbewertungen: evidenzbasierte Versorgung beschleunigen. IQWiG. https://www.iqwig.de/presse/pressemitteilungen/pressemitteilungen-detailseite_89728.html. Accessed 9th March 2023

Remap



Our latest articles:

Are acute therapies and curative drugs more affordable than chronic treatments in rare diseases?

In recent years there has been focus on acute treatments being 'the most expensive drugs in the world'. In 2019, we compared the costs of the most expensive gene/acute therapies and chronic treatments for rare diseases in the US on an annual and lifetime basis to contrast short- and long-term costs.

What are the risks and benefits of Early Access Programs to manufacturers?

For patients with life threatening or seriously debilitating conditions, some countries have schemes which allow patients to have access to the medicine prior to it securing marketing authorisation approval. These schemes are called Early Access Programs (EAPs). There are many examples of EAPs from the early access to medicines scheme (EAMS) in the UK to the special access scheme (SAS) in Australia to the Emergency Access Program (EAP) in South Africa.

How will governments face the cost of medical miracles?

Suhellen Oliveira Da Silva was six months pregnant when she found out that the child she was carrying had the same disease that had left her firstborn paralyzed. But this time, there was a treatment that could make a world of difference. This baby could live a normal life.

The problem was the price: the treatment cost the equivalent of 1.7 million dollars and the public health system in Brazil, where the family lives, refused to pay for it.

Remap

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.

Paul Craddy

MANAGING DIRECTOR
& FOUNDER

Graham Foxon

MANAGING DIRECTOR
& FOUNDER

Remap