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Editorial

This month's medical news brings a wave of promising advancements across various fields. Some of the highlights are as follow:

Drugs: The FDA approved the first interchangeable biosimilar, *Bkemv*, for treating paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uremic syndrome (aHUS). This is a significant step in potentially increasing access and lowering costs for patients with these rare conditions. NICE approved *Setmelanotide* for treating obesity and hyperphagia in specific genetic syndromes. Additionally, *Alemtuzumab* received approval for treating highly active relapsing-remitting multiple sclerosis. *Atogepant* was approved for preventing migraines in adults with frequent attacks. NICE also gave a thumbs up to Selinexor with dexamethasone for treating relapsed or refractory multiple myeloma.

Clinical Research: Studies showed encouraging results for using *Recombinant ADAMTS13* to prevent bleeding episodes in congenital thrombotic thrombocytopenic purpura (TTP) and *Exagamglogene Autotemcel* for treating sickle cell disease. *Aficamten* demonstrated promise in improving exercise capacity for patients with obstructive hypertrophic cardiomyopathy (HCM). Additionally, a study indicated that a continuous *Levodopa-Carbidopa* infusion might offer a safe and effective way to manage motor fluctuations in Parkinson's disease. An analysis from the APROCCHSS trial suggests that *Hydrocortisone plus Fludrocortisone* might reduce mortality in patients with pneumonia-related septic shock.

Devices: NICE approved *Ranibizumab* for treating vision impairment caused by macular oedema. The FDA also cleared Abbott's *Spinal Cord Stimulation (SCS) System* for managing chronic pain.

Treatment Guidelines: NICE issued guidelines on endoscopic duodenal mucosal resurfacing for insulin resistance in type 2 diabetes and image-guided percutaneous laser ablation for liver tumours.

Healthcare Administration: A study highlighted the need for improved communication between surgeons, anaesthesiologists, and primary care providers before surgery, especially for older patients. Research suggests that a standardized electronic template (I-PASS-to-PICU) could improve information exchange during transfers to paediatric intensive care units (PICUs).

This is just a glimpse of the exciting developments in healthcare this month. These advancements offer a ray of hope for patients with various conditions and pave the way for improved treatment options in the future.

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I. Drugs

FDA Approves First Interchangeable Biosimilar for Two Rare Diseases
Source: The FDA, May 2024
Link: https://www.fda.gov/news-events/press-announcements/fda-approves-first-interchangeable-biosimilar-two-rare-diseases

Bkemv as the first interchangeable biosimilar to Soliris (eculizumab) to treat certain rare diseases. Bkemv is approved for the following treatment indications, which are also currently approved for Soliris: the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis; and the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy.

Setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/hst31

Setmelanotide is indicated for 'the treatment of obesity and the control of hunger associated with genetically confirmed Bardet-Biedl syndrome (BBS), loss-of-function biallelic pro-opiomelanocortin (POMC), including PCSK1, deficiency or biallelic leptin receptor (LEPR) deficiency in adults and children 6 years of age and above'.

Alemtuzumab for treating highly active relapsing–remitting multiple sclerosis
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ta312

Alemtuzumab is an antibody that binds to cells of the immune system (B and T cells), causing their destruction. Alemtuzumab has a UK marketing authorisation 'as a single disease modifying therapy in adults with highly active relapsing remitting multiple sclerosis patients with highly active disease despite a full and adequate course of treatment with at least 1 disease modifying therapy.

Atogepant for preventing migraine
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ta973

Atogepant (Aquipta, AbbVie) is indicated for 'prophylaxis of migraine in adults who have at least 4 migraine days per month'.

Selinexor with dexamethasone for treating relapsed or refractory multiple myeloma after 4 or more treatments
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ta970

Selinexor is indicated 'in combination with dexamethasone for the treatment of multiple myeloma in adult patients who have received at least four prior therapies and whose disease is refractory to at least two proteasome inhibitors, two immunomodulatory agents and an anti-CD38 monoclonal antibody, and who have demonstrated disease progression on the last therapy'.

II. Clinical Research

Recombinant ADAMTS13 in Congenital Thrombotic Thrombocytopenic Purpura
Source: The NEJM, May 2024
Link: https://www.nejm.org/doi/full/10.1056/NEJMoa2314793

During prophylaxis with recombinant ADAMTS13 in patients with congenital TTP, ADAMTS13 activity reached approximately 100% of normal levels, adverse events were generally mild or moderate in severity, and TTP events and manifestations were rare.

Exagamglogene Autotemcel for Severe Sickle Cell Disease
Source: The NEJM, May 2024
Link: https://www.nejm.org/doi/full/10.1056/NEJMoa2309676

Treatment with exa-cel eliminated vaso-occlusive crises in 97% of patients with sickle cell disease for a period of 12 months or more.

Aficamten for Symptomatic Obstructive Hypertrophic Cardiomyopathy
Source: The NEJM, May 2024
Link: https://www.nejm.org/doi/full/10.1056/NEJMoa2401424

Among patients with symptomatic obstructive HCM, treatment with aficamten resulted in a significantly greater improvement in peak oxygen uptake than placebo.

Safety and efficacy of continuous subcutaneous levodopa–carbidopa infusion (ND0612) for Parkinson's disease with motor fluctuations (BouNDless): a phase 3, randomised, double-blind, double-dummy, multicentre trial
Source: The Lancet, May 2024
Link: https://www.thelancet.com/journals/laneur/article/PIIS1474-4422(24)00052-8/abstract

ND0612 used in combination with oral immediate-release levodopa–carbidopa increased on time without troublesome dyskinesia and reduced off time, with a favourable benefit–risk profile. ND0612 might offer a safe and efficacious subcutaneous levodopa infusion approach to managing motor fluctuations in people with Parkinson's disease.

Hydrocortisone plus fludrocortisone for community acquired pneumonia-related septic shock: a subgroup analysis of the APROCCHSS phase 3 randomised trial
Source: The Lancet, May 2024
Link: https://www.thelancet.com/journals/lanres/article/PIIS2213-2600(23)00430-7/abstract

In a pre-specified subgroup analysis of the APROCCHSS trial of patients with CAP and septic shock, hydrocortisone plus fludrocortisone reduced mortality as compared with placebo. Although a large proportion of patients with CAP also met criteria for ARDS, the subgroup analysis was underpowered to fully discriminate between ARDS and CAP modifying effects on mortality reduction with corticosteroids

III. Devices

Ranibizumab for treating visual impairment caused by macular oedema secondary to retinal vein occlusion
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ta283/chapter/2-The-technology

By inhibiting the action of VEGF-A, ranibizumab reduces oedema and limits visual loss or improves vision. Ranibizumab has a UK marketing authorisation for 'the treatment of visual impairment due to macular oedema secondary to retinal vein occlusion (branch RVO or central RVO)'.

Abbott Spinal Cord Stimulation (SCS) Systems – P010032/S191
Source: The FDA, May 2024
Link: https://www.fda.gov/medical-devices/recently-approved-devices/abbott-spinal-cord-stimulation-scs-systems-p010032s191

Abbott's Spinal Cord Stimulation (SCS) System is an implantable device, called a neurostimulator, to treat long-term (chronic) pain of the torso, arms, and legs that is difficult to manage (intractable). The Abbott SCS system includes an implantable pulse generator (IPG) and lead wires, as well as the external clinician programmer and patient controller.

IV. Treatment Guidelines

Endoscopic duodenal mucosal resurfacing for insulin resistance in type 2 diabetes
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ipg787

Endoscopic duodenal mucosal resurfacing for insulin resistance in type 2 diabetes. This involves using heat to destroy the lining of the duodenum to encourage a new lining to grow.

Image-guided percutaneous laser ablation for primary and secondary liver tumours
Source: The NICE, May 2024
Link: https://www.nice.org.uk/guidance/ipg788

Image-guided percutaneous laser ablation for primary and secondary liver tumours. This involves heating up liver tissue to destroy tumours.

V. Healthcare Administration

Preoperative Communication Between Anesthesia, Surgery, and Primary Care Providers for Older Surgical Patients
Source: The JCI, May 2024
Link: https://www.jointcommissionjournal.com/article/S1553-7250(24)00032-1/abstract

Surgeons and anesthesiologists infrequently communicate with primary care providers in one rural tertiary center, in contrast to patient expectations and values. These study results will help identify priorities and potentially resolvable barriers to bridging the gap between the inpatient perioperative and outpatient primary care teams. Future studies should focus on strategies to improve communication between hospital and community providers to prevent complications and readmission.

Development and Evaluation of I-PASS-to-PICU: A Standard Electronic Template to Improve Referral Communication for Interfacility Transfers to the Pediatric ICU
Source: The JCI, May 2024
Link: https://www.jointcommissionjournal.com/article/S1553-7250(24)00039-4/abstract

I-PASS-to-PICU was technically feasible, usable, and relevant. The authors plan to further evaluate its effectiveness in improving information exchange during real-time PICU practice.