

Editorial

June 2024 brought a wave of positive developments in the medical field, offering hope to patients battling a variety of diseases. This editorial highlights some of the most significant advancements.

Gene Therapy Takes Center Stage: The FDA's expanded approval of Elevidys, a gene therapy for Duchenne muscular dystrophy (DMD), is a major breakthrough. DMD is a debilitating genetic disease that weakens muscles over time. This therapy offers the potential to slow disease progression and improve quality of life for patients.

Beyond DMD: The news extends beyond DMD. NICE, the UK's health regulator, approved Tafamidis for treating transthyretin amyloidosis with cardiomyopathy, and Voxelotor for treating haemolytic anaemia caused by sickle cell disease. These approvals offer new treatment options for these previously challenging conditions.

Clinical Research Uncovers New Possibilities: Research continues to push boundaries. Studies investigating the use of Dupilumab for eosinophilic esophagitis in children and the potential switch to a new once-daily HIV treatment regimen offer promising results.

Age and Cancer Treatment: The study on endometrial cancer highlights the importance of not overlooking older patients for treatment based solely on age. This finding underscores the need for personalized cancer care.

Weighing Treatment Options: Up-to-date review on Graves' hyperthyroidism provides a valuable resource for healthcare providers and patients alike, outlining the three effective treatment options and considerations for selecting the most appropriate one.

Diagnostics Advance with Point-of-Care Testing: The FDA's approval of the first point-of-care test for hepatitis C infection allows for faster diagnosis and potentially earlier treatment, impacting public health efforts to control the spread of the virus.

Treatment Guidelines Evolve: The updated KDIGO guidelines for chronic kidney disease offer healthcare professionals the latest recommendations for diagnosis, management, and treatment.

Focus on Healthcare Administration: Research on suicide prevention and infection control in long-term care facilities highlights the importance of ongoing efforts to improve patient safety and well-being.

Learning from Experience: The lessons learned from a national hospital antibiotic stewardship program provide valuable insights for other healthcare institutions aiming to implement similar programs and improve antibiotic use.

In conclusion, June's medical news offers a beacon of hope, showcasing advancements in gene therapy, new treatment options, and ongoing research that will ultimately lead to better patient outcomes. The focus on improving healthcare administration practices further strengthens the potential for a healthier future for all.

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I. Drugs

FDA Expands Approval of Gene Therapy for Patients with Duchenne Muscular Dystrophy

Source: The FDA, June 2024

Link: <https://www.fda.gov/news-events/press-announcements/fda-expands-approval-gene-therapy-patients-duchenne-muscular-dystrophy>

Elevidys, a gene therapy for the treatment of Duchenne muscular dystrophy (DMD) for ambulatory and non-ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the DMD gene.

Tafamidis for treating transthyretin amyloidosis with cardiomyopathy

Source: The NICE, June 2024

Link: <https://www.nice.org.uk/guidance/ta984/chapter/2-Information-about-tafamidis>

Tafamidis (Vyndaqel, Pfizer) is indicated for 'the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM)'.

Voxelotor for treating haemolytic anaemia caused by sickle cell disease

Source: The NICE, June 2024

Link: <https://www.nice.org.uk/guidance/ta981/chapter/2-Information-about-voxelotor>

Voxelotor is indicated for 'the treatment of haemolytic anaemia due to sickle cell disease (SCD) in adults and paediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide'.

II. Clinical Research

Dupilumab for Eosinophilic Esophagitis in Patients 1 to 11 Years of Age

Source: The NEJM, June 2024

Link: <https://www.nejm.org/doi/full/10.1056/NEJMoa2312282>

Dupilumab resulted in histologic remission in a significantly higher percentage of children with eosinophilic esophagitis than placebo. The higher-exposure dupilumab regimen also led to improvements in measures of key secondary end points as compared with placebo.

Switch to fixed-dose doravirine (100 mg) with islatravir (0.75 mg) once daily in virologically suppressed adults with HIV-1 on bicittegravir, emtricitabine, and tenofovir alafenamide: 48-week results of a phase 3, randomised, controlled, double-blind, non-inferiority trial

Source: The Lancet, June 2024

Link: [https://www.thelancet.com/journals/lanhiv/article/PIIS2352-3018\(24\)00030-4/abstract](https://www.thelancet.com/journals/lanhiv/article/PIIS2352-3018(24)00030-4/abstract)

Switching to daily doravirine (100 mg) and islatravir (0.75 mg) was non-inferior to bicittegravir, emtricitabine, and tenofovir alafenamide at week 48. However, decreases in CD4 cell and total lymphocyte counts do not support the further development of once-daily doravirine (100 mg) and islatravir (0.75 mg).

Prognostic impact and causality of age on oncological outcomes in women with endometrial cancer: a multimethod analysis of the randomised PORTEC-1, PORTEC-2, and PORTEC-3 trials

Source: The Lancet, June 2024

Link: [https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(24\)00142-6/abstract](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(24)00142-6/abstract)

advanced age was associated with more aggressive tumour features in women with endometrial cancer, and was independently and causally related to worse oncological outcomes. Therefore, our findings suggest that

older women with endometrial cancer should not be excluded from diagnostic assessments, molecular testing, and adjuvant therapy based on their age alone.

Extended pleurectomy decortication and chemotherapy versus chemotherapy alone for pleural mesothelioma (MARS 2): a phase 3 randomised controlled trial

Source: The Lancet, June 2024

Link: [https://www.thelancet.com/journals/lanres/article/PIIS2213-2600\(24\)00119-X/fulltext](https://www.thelancet.com/journals/lanres/article/PIIS2213-2600(24)00119-X/fulltext)

Extended pleurectomy decortication was associated with worse survival to 2 years, and more serious adverse events for individuals with resectable pleural mesothelioma, compared with chemotherapy alone.

Bacille Calmette-Guérin vaccination to prevent febrile and respiratory illness in adults (BRACE): secondary outcomes of a randomised controlled phase 3 trial

Source: The Lancet, June 2024

Link: [https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370\(24\)00195-0/fulltext](https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370(24)00195-0/fulltext)

In contrast to the beneficial off-target effects reported following neonatal BCG in infants, a small increased risk of symptomatic febrile or respiratory illness was observed in the 12 months following BCG vaccination in adults. There was no evidence of a difference in the risk of severe disease.

Effects of sintilimab plus chemotherapy as first-line treatment on health-related quality of life in patients with advanced esophageal squamous cell carcinoma: results from the randomized phase 3 ORIENT-15 study

Source: The Lancet, June 2024

Link: [https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370\(24\)00202-5/fulltext](https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370(24)00202-5/fulltext)

The statistically significant differences of several HRQoL scales and improvements in delayed deterioration observed in our study further support the use of sintilimab plus chemotherapy as first-line treatment for advanced ESCC.

Selection of therapy for Graves' hyperthyroidism

Source: The Uptodate, June 2024

Link: <https://www.uptodate.com/contents/whats-new-in-primary-care>
https://www.uptodate.com/contents/graves-hyperthyroidism-in-nonpregnant-adults-overview-of-treatment?sectionName=Treatment+options&topicRef=8357&anchor=H1095594915&source=see_link#H1095594915

There are three effective treatment options for Graves' disease: antithyroid drugs (thionamides), radioiodine, and surgery (table 2). Selection of therapy is generally based upon individual patient factors (eg, severity of hyperthyroidism, older age) as well as patient values and preferences. In a 2023 global survey of endocrinologists, 91.5 percent of respondents preferred antithyroid drugs, 7 percent radioiodine, and 1.5 percent surgery

III. Devices

FDA Permits Marketing of First Point-of-Care Hepatitis C RNA Test

Source: The FDA, June 2024

Link: <https://www.fda.gov/news-events/press-announcements/fda-permits-marketing-first-point-care-hepatitis-c-rna-test>

Cepheid for the Xpert HCV test and GeneXpert Xpress System, the first hepatitis C virus (HCV) test that can be used to bring diagnosis to appropriately certified point-of-care settings for individuals at risk for hepatitis C.

IV. Treatment Guidelines

The 2024 Kidney Disease: Improving Global Outcomes (KDIGO) guidelines for chronic kidney disease

Source: The Uptodate, June 2024

Link: <https://www.uptodate.com/contents/whats-new-in-nephrology-and-hypertension>
https://www.uptodate.com/contents/overview-of-the-management-of-chronic-kidney-disease-in-adults?topicRef=8352&source=see_link

The Kidney Disease: Improving Global Outcomes (KDIGO) guidelines are updated periodically with new or revised recommendations on the evaluation, diagnosis, and management of chronic kidney disease (CKD). The recommendations for the use of new equations to estimate glomerular filtration rate (GFR) using creatinine and cystatin C, risk estimates according to CKD staging using these new equations, and recommendations for the use of sodium-glucose cotransporter-2 (SGLT2) inhibitors and nonsteroidal mineralocorticoid receptor antagonists.

V. Healthcare Administration

Evaluating the Prevalence of Four Recommended Practices for Suicide Prevention Following Hospital Discharge

Source: The JCI, June 2024

Link: [https://www.jointcommissionjournal.com/article/S1553-7250\(24\)00067-9/fulltext](https://www.jointcommissionjournal.com/article/S1553-7250(24)00067-9/fulltext)

The study revealed a significant gap in implementation of recommended practices related to prevention of suicide postdischarge. Additional research is needed to identify factors contributing to this implementation gap.

Infection Control Measure Performance in Long-Term Care Hospitals and Their Relationship to Joint Commission Accreditation

Source: The JCI, June 2024

Link: [https://www.jointcommissionjournal.com/article/S1553-7250\(24\)00057-6/fulltext](https://www.jointcommissionjournal.com/article/S1553-7250(24)00057-6/fulltext)

Although this study was not designed to establish causality, positive associations were observed between Joint Commission accreditation and CLABSI and CAUTI measures, and Joint Commission–accredited LTCHs attained more consistent high performance over the four-year study period for all three measures. Influencing factors may include the focus of Joint Commission standards on infection control and prevention (ICP), including the hierarchical approach to selecting ICP–related standards as inputs into LTCH policy.

Lessons Learned from a National Hospital Antibiotic Stewardship Implementation Project

Source: The JCI, June 2024

Link: [https://www.jointcommissionjournal.com/article/S1553-7250\(24\)00101-6/abstract](https://www.jointcommissionjournal.com/article/S1553-7250(24)00101-6/abstract)

Lessons learned from the Safety Program can be applied by other teams looking to promote an effective ASP at their hospital or system. The themes that emerged in this study likely also have relevance across a wide range of large-scale quality improvement initiatives.