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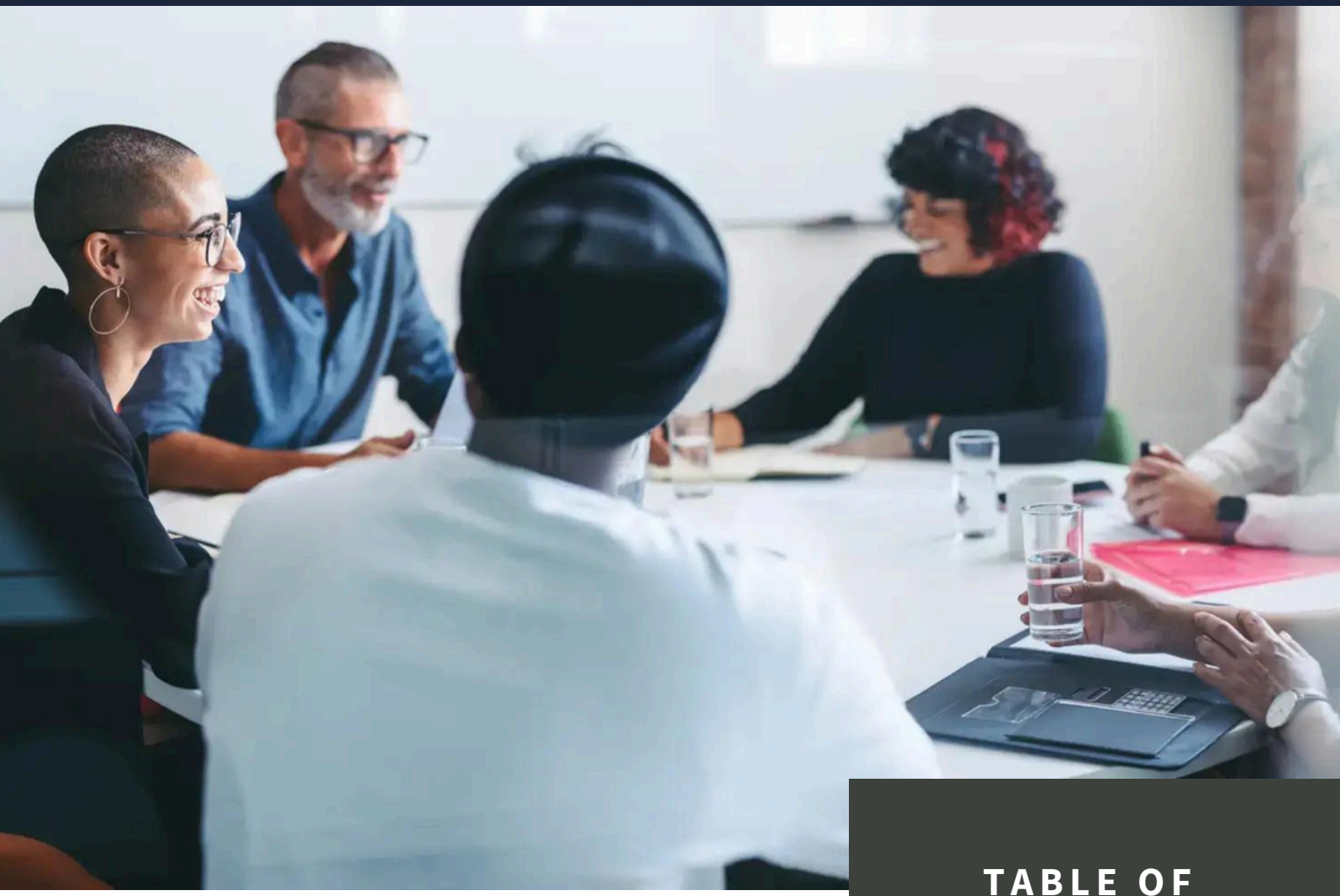

EMA AND FDA COLLABORATION ON INDUSTRY BEST PRACTICES

We are excited to announce that the European Medicines Agency (EMA) and the US Food and Drug Administration (FDA) have collaboratively released a comprehensive Q&A document, detailing their perspectives on various Quality and Good Manufacturing Practice (GMP) aspects associated with PRIME/Breakthrough therapy applications.

The PRIME initiative by EMA and the breakthrough therapy designation program by FDA are tailored to accelerate the development of innovative medicinal products that target unmet medical needs. [1]

Continue reading here.

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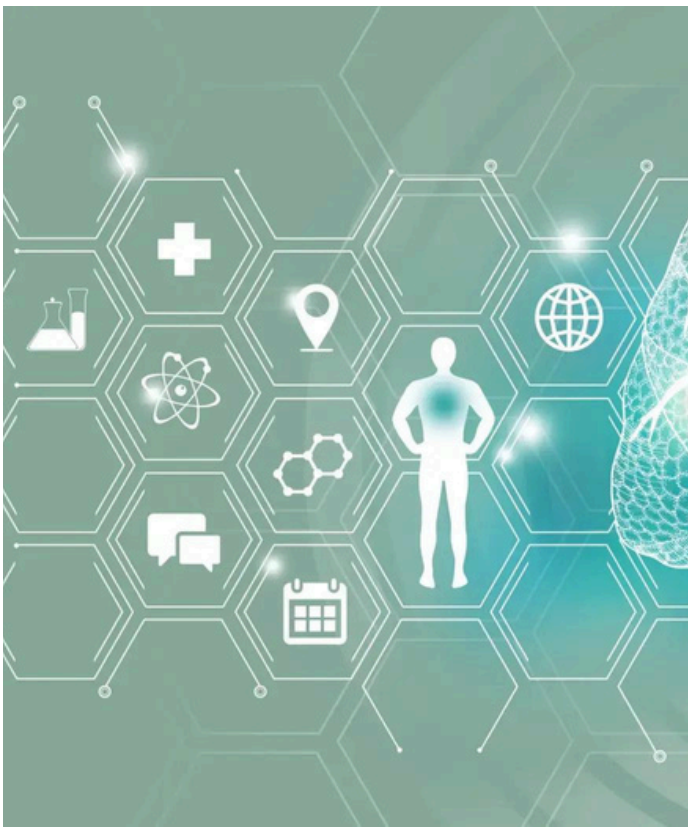
FDA: NOVEL DRUG APPROVALS FOR 2023

New drugs and therapeutic biological products often signify novel treatment options and advancements in healthcare for the American population. Each year, CDER shares a complete list of all novel drug approvals for the prior year. The FDA's Center for Drug Evaluation and Research (CDER) plays a crucial role in providing clarity to drug developers regarding study design elements and required data in drug applications, ensuring a comprehensive assessment. This process hinges on CDER's deep understanding of the underlying science, testing and manufacturing protocols, and the specific diseases and conditions targeted by these new products. Within this framework, certain drugs are designated as new molecular entities (NMEs) for FDA review, representing compounds with active moieties not previously approved by the agency, offering promising new therapies.

While some NMEs may contain active moieties closely related to previously approved products, the FDA's classification of drugs as NMEs for review purposes remains distinct from its determination of whether a drug qualifies as a "new chemical entity" (NCE) under the Federal Food, Drug, and Cosmetic Act.[2]

Access the complete list of novel drug approvals for 2023 here.

UNDERSTANDING TUBERCULOSIS RISK AMONG HOUSEHOLD CONTACTS: INSIGHTS FROM BRAZIL



Although it is well-known that household contacts of tuberculosis patients are highly susceptible to contracting the disease, there is a dearth of published evidence focusing on this high-risk group within low-income and middle-income countries. This study aimed to fill this gap by utilizing nationwide data from Brazil to estimate the incidence of tuberculosis and explore the socioeconomic and clinical factors influencing its occurrence among a cohort of tuberculosis patient contacts.

In this cohort investigation, researchers linked individual socioeconomic and demographic information from the 100 Million Brazilian Cohort to mortality records and tuberculosis registries. Continue reading here.[3]

FDA APPROVES REVOLUTIONARY THERAPY FOR SICKLE CELL DISEASE

The long-awaited announcement came on December 8th, revealing that the US Food and Drug Administration (FDA) had given the green light to two gene therapies for sickle cell disease. This milestone includes the groundbreaking approval of the first CRISPR-based treatment for any disorder. The treatments, known as exagamglogene autotemcel (exa-cel) and lovotibeglogene autotemcel (lovocel), are now authorized for individuals aged 12 years and older who have a history of vaso-occlusive events.

For individuals with sickle cell disease, these genetic therapies offer hope for transformation, particularly as the disease disproportionately affects Black individuals, who have historically faced neglect and discrimination.

This approval marks a significant achievement for both science and medicine, arriving just over a decade after the initial discovery of CRISPR. Read more here.[4]

EMA RECOMMENDS GROUNDBREAKING APPROVAL OF CRISPR/CAS9 THERAPY

The European Medicines Agency (EMA) has given its recommendation for the approval of a groundbreaking medicine utilizing CRISPR/Cas9, an innovative gene-editing technology to modify the patient's own blood stem cells. Known as Casgevy (exagamglogene autotemcel), this treatment is designed for individuals aged 12 and above suffering from transfusion-dependent beta thalassemia and severe sickle cell disease. It targets patients for whom traditional hematopoietic stem cell transplantation is suitable but a compatible donor is not available.[5]

Continue reading here.

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Thanks for reading!

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